

# Exhibit A



*Office of Industrial Affairs*

March 4, 2005

*via United Parcel Service*

Patrick Girondi  
Errant Gene Therapeutics, LLC  
141 W. Jackson Boulevard  
Suite 300  
Chicago, Illinois 60604

Re: - License Agreement between SKI and Errant Gene Therapeutics, LLC  
- SKI Ref: SK#9927


Dear Pat:

Please find enclosed two copies of the above referenced agreement for your signature. Please send a fully executed to my attention.

Once I received a fully executed copy of the agreement, I will send an invoice for incurred patent expenses with copies of the attorney bills including the outstanding invoice for \$17,039.57 dated Oct. 18, 2004 for patent expenses incurred from Feb. 1, and Sept. 30, 2004.

For patent prosecution related matters, I will instruct our attorney to copy Lisa Wilson on any correspondence. Let me know if you want copies sent to anyone else. Thanks.

Sincerely,

  
Viviane

Encl.

*Memorial Sloan-Kettering Cancer Center  
1275 York Avenue, New York, New York 10021  
Telephone 212.639.6181 • FAX 212.717.3439  
NCI-designated Comprehensive Cancer Center*

## EXCLUSIVE LICENSE AGREEMENT

for SKI's technology

"SK 972"

(SK#9927)

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## LICENSE AGREEMENT

This License Agreement (the "Agreement") is effective on the date last subscribed below (the "Effective Date"), and is by and between **Sloan-Kettering Institute for Cancer Research** (hereinafter referred to as "SKI"), a New York membership corporation with principal offices at 1275 York Avenue, New York, New York 10021, and **Errant Gene Therapeutics, LLC**, a limited liability company formed under the laws of the State of Delaware with principal offices located at 141 W. Jackson Blvd., Suite 300, Chicago, Illinois 60604 ("LICENSEE").

### WITNESSETH

WHEREAS, SKI is the sole owner of certain Patent Rights (as defined herein) developed during the course of research conducted by Dr. Michel Sadelain (hereinafter "Inventor") and assigned to SKI, and SKI has the right to grant licenses under said Patent Rights; and

WHEREAS, SKI desires to have the Patent Rights utilized in the public interest and is willing to grant a license to its interest thereunder; and

WHEREAS, LICENSEE seeks to commercially develop the Patent Rights through a thorough and diligent program of exploiting the Patent Rights whereby public utilization shall result.

NOW, THEREFORE, in consideration of the premises and the mutual covenants contained herein, the parties hereto agree as follows:

### ARTICLE I — DEFINITIONS

For the purpose of this Agreement, the following words and phrases shall have the following meanings:

1.0 "Additional Data" means data as defined in Exhibit A.

1.1 "Entity" means any individual, corporation, limited liability company, partnership, joint venture, association, trust or unincorporated organization or other entity or a government or any agency or political subdivision thereof.

1.2 "Field A" shall mean use of the Patent Rights in the field of research and treatment of thalassemia.

1.3 "Field B" shall mean use of the Patent Rights in the field of research and treatment of hemoglobinopathies (including sickle cell disorder) other than thalassemia.

1.4 "Field of Use" shall mean, individually and collectively, Field A and Field B.

1.5 "Field A Licensed Product" shall mean any Licensed Product intended for application in Field A.

1.6 "Field B Licensed Product" shall mean any Licensed Product intended for application in Field B.

1.7 "Licensed Product" shall mean any process, service, or any product or part thereof made, leased, used or sold by or on behalf of LICENSEE and which:

- (a) is covered in whole or in part by a Valid Claim of the Patent Rights in the country in which the product or part thereof is made, leased, used or sold; or
- (b) is manufactured by using a process covered in whole or in part by a Valid Claim of the Patent Rights in the country of manufacture.

1.8 "LICENSEE" shall include "Affiliates", where "Affiliates" shall mean, any Entity controlling, controlled by, or under common control with a party hereto. The term "control" wherever used throughout this Agreement shall mean ownership, directly or indirectly, of more than 50% of the equity capital. With regard to SKI, "Affiliate" shall mean the Memorial Sloan-Kettering Cancer Center and the Memorial Hospital for Cancer and Allied Diseases.

1.9 "Net Sales" shall mean revenues earned by LICENSEE or any Sublicensee from any bona fide transaction for which consideration is received or expected for the sale, use, lease, transfer or other disposition of Licensed Products, less the sum of the following:

- (a) Discounts allowed in amounts customary in the trade;
- (b) Sales, tariff duties and/or use taxes directly imposed and with reference to particular sales;
- (c) Outbound transportation prepaid or allowed;
- (d) Amounts allowed or credited on returns, rebates, recalls, or retroactive price reductions;
- (e) Bad debts and uncollectible receivables provided that, in any calendar year, such deduction will not exceed four percent (4%) of the total billings for sales of Licensed Products sold in that year.

No deductions shall be made for commissions paid to individuals whether they be with independent sales agencies or regularly employed by LICENSEE and on its payroll, or for cost of collections.

A sale of Licensed Product shall be deemed completed at the time LICENSEE or its Sublicensee invoice for such Licensed Products. In the event that LICENSEE or any Sublicensee sells a Licensed Product in combination with other active ingredients or components which are not Licensed Products ("Other Items"), the Net Sales for purposes of payments on the combination shall be calculated on the basis of the following:

- (f) If all Licensed Products and Other Items contained in the combination are available separately, the Net Sales will be calculated by multiplying the Net Sales of the combination by the fraction  $A/(A+B)$ , where A is the separately available price of all Licensed Products in the combination, and B is the separately available price for all Other Items in the combination.
- (g) If the combination includes Other Items which are not sold separately (but all Licensed Products contained in the combination are available separately), the Net Sales for purposes of royalty payments will be calculated by multiplying the Net Sales of the combination by  $A/C$ , where A is as defined above, and C is the invoice price of the combination.
- (h) If the Licensed Products contained in the combination are not sold separately, the parties agree that the Net Sales for such combination shall be an amount determined by reducing the Net Sales as would otherwise be applicable on the basis of the first sentence of this paragraph 1.9 ((a) through (g)), so that the amount of the Net Sales, as so adjusted, will reflect the fair value that the Licensed Product attributed to the overall product sold.
- (i) The term "Other Items" does not include solvents, diluents, carriers, excipients, buffers or the like used in formulating a product.

1.10 "Patent Rights" shall mean all of the following SKI intellectual property:

- (a) the United States and foreign patents and patent applications listed in Exhibit A;
- (b) the United States and foreign patents issued from the applications listed in Exhibit A and from divisionals and continuations of these applications;
- (c) claims of U.S. and foreign continuation-in-part applications, and of the resulting patents, which are directed to subject matter specifically described in the U.S. and foreign patent applications listed in Exhibit A;
- (d) any reissues or re-examinations of patents described in (a), (b), or (c), above.

1.11 "Royalty Year" shall mean each twelve month period commencing January 1 and ending December 31 during the term of this Agreement. For the first year of this Agreement, the Royalty Year shall be the period of time between the signing of the Agreement and December 31.

1.12 "Sublicensee" means a person other than an Affiliate of LICENSEE to which LICENSEE grants sublicense rights under the license granted to LICENSEE hereunder, provided, however, that third parties that are permitted to manufacture or finish Licensed Products for supply to or as directed by LICENSEE or its Affiliates, or to or as directed by any Sublicensee or its Affiliates, but which are not allowed to sell, use, lease and, import and otherwise dispose of Licensed Products, are not

"Sublicensees" hereunder. "Sublicense" or "sublicense" shall have the correlative meaning, that is, an agreement, or the making of an agreement that sets forth the terms under which LICENSEE agrees that a third party may become a Sublicensee hereunder.

1.13 "Valid Claim" means, individually and collectively, a Valid Issued Claim and a Valid Pending Claim.

1.14 "Valid Issued Claim" means an unexpired claim of any issued patent within the Patent Rights, so long as such claim shall not have been held to be invalid or unenforceable in an unappealable decision of a court or other authority of competent jurisdiction.

1.15 "Valid Pending Claim" means a pending claim of a patent application within the Patent Rights, so long as such claim shall not (i) have been held to be invalid or unenforceable in an unappealable decision of a court or other authority of competent jurisdiction, (ii) have been finally rejected by the relevant patent office and such rejection can no longer be subject to proceedings of a court or other authority of competent jurisdiction or (iii) have been irrevocably abandoned.

## ARTICLE II - GRANT

2.1 SKI hereby grants to LICENSEE the exclusive worldwide right and license under the Patent Rights, including the right to sublicense, to practice the methods embodied by the Patent Rights to make, have made, use, lease and sell, import and otherwise dispose of Licensed Products, in the Field of Use, subject to the rights reserved or observed in Section 2.2 below.

2.2 Notwithstanding any other provisions of this Agreement, it is agreed that SKI and its Affiliates shall retain the right to practice the Patent Rights licensed hereunder, for its own teaching, non-commercial research and educational purposes. Further, SKI reserves the right to permit qualified researchers at governmental or research institutions to use the Patent Rights in the Field of Use solely for noncommercial research purposes. Such uses shall not be considered as infringement. In addition, all rights reserved to the United States Government and others under 35 USC §§ 200-212, as amended, shall remain and shall in no way be affected by this Agreement. To that end, and insofar as may be required thereby, LICENSEE agrees that any Licensed Product used and sold in the United States will be manufactured substantially in the United States.

2.3 LICENSEE hereby agrees that every Sublicense to which it is a party, and which shall relate to the rights, privileges and license granted hereunder shall contain a statement describing the date upon which LICENSEE'S exclusive rights, privileges and license hereunder shall terminate.

2.4 LICENSEE agrees that Sublicensees shall not further enter into Sublicenses. LICENSEE agrees that any Sublicense granted by it shall provide that the obligations to SKI of Sections 3.1, 8.1, 8.2, 8.4 and 12.8, and Articles V, IX and X, XII, and insofar as applicable to the foregoing, Article XV, of this Agreement shall be binding upon the Sublicensee as if it were a party to this Agreement. LICENSEE further agrees to attach copies of those Articles to each Sublicense.

2.5 LICENSEE may not enter into a Sublicense with a third party without SKI's consent; such consent shall not be unreasonably withheld. Any Sublicense approval not denied by SKI for good reason within ten (10) business days after receipt of request therefore in writing shall be deemed approved. LICENSEE agrees to forward to SKI a copy of any and all fully executed Sublicenses, and further agrees to timely forward to SKI a copy of such reports received by LICENSEE from its Sublicensees during the preceding Royalty Year.

2.6 If LICENSEE receives from Sublicensees anything of value in lieu of cash payments based upon payment obligations of any Sublicense under this Agreement, insofar as such payments relate to the grant to the Sublicensee of a Sublicense in respect of the Patent Rights, LICENSEE shall pay SKI royalty or other payments as required by Article 4, based on the fair market value of such payment, unless SKI waives in writing such payment obligation. The foregoing sentence shall not be applied to any amounts paid by a Sublicensee to LICENSEE for purchase of stock or other equity interests in the LICENSEE in connection with any Sublicense, except to the extent such monies are paid to LICENSEE as a substitute, wholly or in part, for a royalty on sales of Licensed Product or for license initiation, maintenance or other related fees in respect of which payment would otherwise be due hereunder, nor shall the first sentence of this Section 2.6 apply to any amounts paid to the LICENSEE by any Sublicensee for, or to the delivery or providing by Sublicensee to LICENSEE of, bona fide product development or research work, or clinical studies or results thereof.

2.7 The license granted hereunder shall not be construed to confer any rights upon LICENSEE by implication, estoppel or otherwise as to any technology not included in the Patent Rights; nor shall LICENSEE have any royalty obligations beyond the Patent Rights and the technology expressly included therein.

2.8 In addition to and separate from the grant of the license hereunder in respect of the Patent Rights, SKI agrees to provide and deliver to the LICENSEE, promptly following the signing of this Agreement, and as and when the same becomes available, as applicable, the data and other deliverables, and instruction, described on Exhibit B hereto (the "Additional Data"). LICENSEE shall be free to use, employ and disclose, and to allow others to use, employ and disclose, the Additional Data as it sees fit during the term and subsequent to the expiration of this Agreement, provided that such use does not represent an unlicensed infringement of the Patent Rights and provided that such disclosure complies with Article XIV.



ARTICLE III - DUE DILIGENCE

3.1 LICENSEE and its Sublicensees shall use commercially reasonable efforts to develop and seek registration for, and to introduce into the market, Licensed Products in Field A and in Field B through an appropriately thorough and diligent program for exploitation of the Patent Rights as detailed in the Plan referred to below, consistent with sound and reasonable scientific and business judgment, and thereafter continue active, diligent marketing efforts for one or more Licensed Products throughout the life of this Agreement.

3.2 In furtherance of Section 3.1 above, LICENSEE shall use commercially reasonable efforts to adhere to the following milestone: LICENSEE has delivered to SKI, prior to the Effective Date of this Agreement, a draft of its business, research, and development plan, and within ninety (90) days after the Effective Date, LICENSEE will deliver a revised, reasonably comprehensive business, research, and development plan and schedule to reach development milestones (the "Plan"), including, for example, to the extent applicable, relevant schedules of investments contemplated to be needed to implement the Plan, financial, equipment, facility plans, number and kind of personnel and time planned for each phase of development of the Licensed Products for a three year period, to the extent formed by LICENSEE. The Plan will include the following milestones (with it being acknowledged that the ability of LICENSEE to meet these milestones is predicated on numerous variables and conditions some of which being beyond the control of LICENSEE): the filing of an Investigational New Drug (IND) application for a Field A Licensed Product shall occur within nine (9) months of the Effective Date, the enrollment of the first patient in a Phase I/II clinical trial shall occur within the first anniversary of the Effective Date, the enrollment of the first patient in a pivotal clinical trial shall occur by the third anniversary of the Effective Date, NDA submission shall occur by the fourth anniversary of the Effective Date, preclinical work in Field B shall start within a year of the Effective Date. Similar reports shall be provided to SKI annually to relay update and status information on LICENSEE's business, research and development progress relating to the Licensed Products, including projections of activity anticipated for the next reporting year.

3.3 In the event that SKI reasonably determines as respects either Field A or Field B that LICENSEE is not performing in accordance with Section 3.1 and 3.2 above, SKI will advise LICENSEE accordingly in writing and describe in reasonable detail the basis for the determination, and provide LICENSEE with a reasonable opportunity (not less than three (3) months) to implement measures in order to address the concerns identified by SKI. LICENSEE's failure to address and to resolve such SKI concerns in a timely manner as respects Field A or Field B, as the case may be, shall be grounds for SKI to terminate the Agreement pursuant to Section 12.4 as respects the applicable field.

3.4 LICENSEE will endeavor to sponsor the first clinical trial relating to the first Field A Licensed Product, conducted for the first time in the United States, at SKI. However, SKI is not obligated to conduct such clinical trial.

ARTICLE IV -PAYMENTS

4.1 For the rights, privileges and licenses granted hereunder, LICENSEE shall pay to SKI, in the manner hereinafter provided, until the end of the last to expire patent of the Patent Rights or until this Agreement shall be terminated as hereinafter provided, whichever occurs first:

- (a) A license issue fee of sixty thousand (\$60,000), payable as follows:
  - (i) twenty thousand dollars (\$20,000) payable upon signing of the Agreement;
  - (ii) twenty thousand dollars (\$20,000) payable within six (6) months of signing of this Agreement; and
  - (iii) twenty thousand dollars (\$20,000) payable within one (1) year of signing of this Agreement.
- (b) Commencing on the first day of the first Royalty Year following the second anniversary of the Effective Date, an annual minimum payment of twenty thousand dollars (\$20,000) per Royalty Year. Payments made under this Section 4.1(b) shall be deducted from payments due to SKI under Sections 4.1(c) of this Agreement in the applicable Royalty Year, that is, payments made under this Section 4.1(b) shall be in the nature of a minimum royalty and shall be applied to, and as an offset against, royalty obligations otherwise arising for the ensuing Royalty Year pursuant to Section 4.1(c) below.
- (c) A royalty in an amount equal to three percent (3%) of the Net Sales by LICENSEE or any Sublicensee of any Field A Licensed Product covered in whole or in part by a Valid Issued Claim, or two percent (2%) of the Net Sales by LICENSEE or any Sublicensee of any Field A Licensed Product not covered by a Valid Issued Claim, but instead covered by a Valid Pending Claim.  
  
A royalty in an amount equal to six percent (6%) of the Net Sales by LICENSEE or any Sublicensee of any Field B Licensed Product covered in whole or in part by a Valid Issued Claim, or four percent (4%) of the Net Sales by LICENSEE or any Sublicensee of any Field B Licensed Product not covered by a Valid Issued Claim, but instead covered by a Valid Pending Claim.
- (d) In the event that LICENSEE or any Sublicensee shall determine it to be necessary or appropriate under the circumstances to obtain any additional license(s) in order to commercialize Licensed Products and the licensor(s) in respect of such additional licenses require LICENSEE or the Sublicensee to make payments in consideration for such license(s), then up to thirty percent (30%) of

such third party royalty obligations may be deducted from the royalty amount otherwise payable to SKI under this Section 4.1(c) until such royalty amount is reduced to, but not less than, seventy percent (70%) of what it otherwise would have been had such additional license(s) not been required. Notwithstanding the above if such additional licenses require LICENSEE or the Sublicensee to make payments in consideration for such license(s) on Other Items as defined in Section 1.7, then such third party royalty obligations shall not be deducted from the royalty amount otherwise payable to SKI under Section 4.1(c) above.

(e) Milestone payments as follows:

(i) \$100,000 upon dosing of first patient with a Field A Licensed Product for a pivotal clinical trial for submission to the United States Food and Drug Administration ("US FDA"), or within thirty (30) days after the end of the calendar year 2008, which ever is earlier;

(ii) \$200,000 upon receipt of the first New Drug Application (NDA) approval from the US FDA for any Field A Licensed Product;

(iii) \$200,000 upon receipt of the first marketing approval in the European Union for any Field A Licensed Product;

(iv) \$50,000 upon receipt of every marketing approval in any other country for any Field A Licensed Product where payment is not otherwise required above upon receipt of such approval;

(v) \$300,000 upon dosing of first patient with a Field B Licensed Product for a pivotal clinical trial for submission to the US FDA, or within thirty (30) days after the end of the calendar year 2008, which ever is earlier;

(vi) \$1,000,000 upon receipt of the first NDA approval from the US FDA for any Field B Licensed Product;

(vii) \$750,000 upon receipt of the first marketing approval in the European Union for any Field B Licensed Product; and

(viii) \$200,000 upon receipt of every marketing approval in any other country for any Field B Licensed Product where payment is not otherwise required above upon receipt of such approval;

LICENSEE shall have the right and option to satisfy each payment obligation accruing under subparagraph (i) through (iv) above through payments made in cash in four (4) equal quarterly

installments, commencing on the last day of March, June, October, or December following the occurrence of the event giving rise to such payment.

- (f) In addition, LICENSEE shall pay SKI twenty-five percent (25%) of payments that is not subject to the Royalty under 4.1(c) received by LICENSEE from a Sublicensee in consideration for the grant of a Sublicense, e.g., but not limited to, up-front licensing fees, maintenance fees, milestones; provided, however, that excluded from amounts in respect of which payments would otherwise be required under this subparagraph (f) are: debt/loans, documented payments for sponsored or other research or development funding specifically committed and actually utilized to developed Licensed Products in the Field of Use; amounts paid to LICENSEE for bona fide product development or research work or clinical studies or results thereof and any amounts paid for the purchase of stock or other equity interests in LICENSEE except to the extent such monies are paid to LICENSEE as a substitute, wholly or in part, for a royalty on sales of Licensed Product or for license initiation, maintenance or other related fees.

4.2 No multiple royalties shall be payable because any Licensed Product, or its manufacture, use, lease or sale, are or shall be covered by more than one of the individual patents, patent rights or other items included among the Patent Rights licensed under this Agreement.

4.3 Royalty payments shall be paid in United States dollars in New York, NY, or at such other place as SKI may reasonably designate consistent with the laws and regulations controlling in any foreign country, but not in any other currency. Royalty payments for transactions outside the United States of America shall first be determined in the currency of the country in which they are earned, and then converted to United States dollars. If any currency conversion shall be required in connection with the payment of royalties hereunder, such conversion shall be made by using the exchange rate prevailing at the Chase Manhattan Bank (N.A.) on the last business day of the calendar quarterly reporting period to which such royalty payments relate. Notwithstanding the foregoing, if the law or regulation of any country shall at any time operate or impede the transfer of the funds therefrom to the United States, LICENSEE shall have right to pay or cause to be paid, royalties hereunder on account of its sales and the sales of its Affiliates and Sublicensees in such country, by depositing local currency to the account of SKI in an interest bearing account at the prevailing commercial interest rate in a bank in such country, and notifying SKI to such effect. LICENSEE shall thereafter cooperate with SKI in SKI's efforts to obtain the lawful release of such funds to SKI. Any and all loss of exchange value, taxes, or other expenses incurred in the transfer or conversion of foreign currency into U.S. dollars, and any income, remittance, or other taxes on such royalties required to be withheld at the source shall be the exclusive responsibility of LICENSEE. Royalty statements shall

show sales both in the local currency and US dollars, with the exchange rate used clearly stated.

Any payment due by LICENSEE shall be made quarterly, within thirty (30) days after March 31, June 30, October 31, or December 31. If no payment is due, it shall be so notified.

#### 4.4 Interest

- (a) LICENSEE shall pay to SKI interest on any amounts not paid when due. Such interest will accrue from the fifteenth (15<sup>th</sup>) day after the payment was due at a rate two percent (2%) above the daily prime interest rate, as determined by The Chase Manhattan Bank (N.A.) or its successor entity, on each day the payment is delinquent, and the interest payment will be due and payable on the first day of each month after interest begins to accrue, until full payment of all amounts due SKI is made.
- (b) SKI's rights to receive such interest payments shall be in addition to any other rights and remedies available to SKI.
- (c) If the interest rate required in this Subsection exceeds the legal rate in a jurisdiction where a claim for such interest is being asserted, the required interest rate shall be reduced, for such claim only, to the maximum interest rate allowable in the jurisdiction.

### ARTICLE V - REPORTS AND RECORDS

5.1 LICENSEE shall keep full, true and accurate books of account containing all particulars that may be necessary for the purpose of showing the amounts payable to SKI hereunder. Said books and records shall be maintained for a period of no less than five (5) years following the period to which they pertain. For the term of this Agreement, upon reasonable written notice, LICENSEE shall allow SKI or its agents to inspect such books and records for the purpose of verifying LICENSEE'S royalty statement or compliance in other respects with this Agreement. Such inspections shall be during normal working hours of LICENSEE. Should such inspection lead to the discovery of a greater than ten percent (10%) discrepancy in reporting to SKI's detriment, LICENSEE agrees to pay the full cost of such inspection.

5.2 LICENSEE, within thirty (30) days after March 31, June 30, October 31, and December 31 of each year following the initial commercial sale or application of Licensed Product, shall deliver to SKI true and accurate reports, giving such particulars of the business conducted by LICENSEE and its Sublicensees during the preceding three-month period under this Agreement as shall be pertinent to a royalty accounting hereunder. These shall include at least the following, to be itemized per Licensed Product:

- (a) Number of Licensed Products commercially used, manufactured and sold, rented or leased.
- (b) Total billings for Licensed Products commercially used, sold, rented or leased detailing products name, country of manufacture, country where sold, units sold, prices of each products.
- (c) Deductions applicable as provided in Paragraph 1.9.
- (d) Total royalties due by Licensee for Products sale detailing and identifying each applicable deductions.
- (e) Names and addresses of all Sublicensees of LICENSEE.
- (f) Total royalty generating revenues of Sublicensees hereunder, including the identification of each such Sublicensee, the corresponding revenue amounts, and identification of the corresponding Licensed Product.
- (g) Total Sublicensing fee income, showing detailed calculation such amount the itemized applicable deductions as provided in 4.1.

5.3 With each such report submitted, LICENSEE shall pay to SKI the royalties due and payable under this Agreement. Notwithstanding the foregoing, if and in the event that payment to LICENSEE or any Sublicensee is delayed because of governmental reimbursement delays, SKI will, in the spirit of cooperation, allow for a corresponding delay in the royalty payments hereunder. If no royalties shall be due, LICENSEE shall so report.

#### ARTICLE VI - PATENT PROSECUTION

6.1 Unless otherwise directed by LICENSEE and in any event subject to application of the last sentence of Section 6.3 below, SKI agrees to use all commercially reasonable and diligent efforts to pursue, and to cause patent counsel to pursue, the issuance of, and to maintain, and to cause patent counsel to maintain, the patents within the Patent Rights.

6.2 LICENSEE shall be responsible for and pay all past and future costs and expenses reasonably incurred by SKI for the preparation, filing, prosecution, issuance, and maintenance of the Patent Rights; provided, however, that SKI hereby represents that the costs thereof to date (which shall be reimbursed by LICENSEE to SKI within thirty (30) days after the date hereof) do not exceed \$36,000. Notwithstanding the foregoing, if and in the event that the Patent Rights should be licensed to others outside of the Field of Use, as a condition to any such license, SKI will require that the patent costs and expenses incurred in respect of the Patent Rights in the future, be equitably allocated among such licensee and the LICENSEE hereunder. In addition, to the extent that the patent costs and expenses incurred and paid for by LICENSEE prior to the grant of any such license inure to the benefit of any other such license, SKI will account for such benefit in entering into the subsequent license and will afford to LICENSEE a corresponding and equitably determined credit against future payment obligations of LICENSEE hereunder, such that the ultimate responsibility for all prior

and subsequent patent costs and expenses will be borne equitably by all licensees under the Patent Rights.

6.3 SKI shall allow LICENSEE an opportunity to participate in the prosecution of the Patent Rights. LICENSEE or its patent counsel may directly contact SKI's patent counsel, and SKI shall entertain and consider all reasonable comments and suggestions made by LICENSEE or its own patent counsel regarding the prosecution of the PATENT RIGHTS. Nevertheless, SKI and its patent counsel will be solely responsible for the filing, prosecuting and maintaining of all patents and patent applications included in the Patent Rights. SKI shall keep LICENSEE informed and apprised of the continued prosecution and shall provide LICENSEE with copies of relevant documents. LICENSEE agrees to keep this documentation confidential. In no event, however, shall the involvement of LICENSEE or its counsel in the patent prosecution process have the effect of relieving SKI from its responsibilities for the patent prosecution, or impose any obligations on LICENSEE in that regard. In any country where LICENSEE elects not to have a patent application filed or to pay expenses associated with the filing, prosecuting or maintaining of patent application or patent, LICENSEE shall have no obligation to do so, but SKI may file, prosecute and/or maintain the patent application or patent at its own expense and for its own benefit and the license hereunder shall, to the extent of such refusal, exclude such countries.

#### ARTICLE VII - INFRINGEMENT

7.1 LICENSEE, as the exclusive commercial user in the Field of Use of the Patent Rights, shall assume primary responsibility for enforcing the Patent Rights within relevant commercial markets in the Field of Use so long as this Agreement shall remain in effect. In addition, so long as this Agreement shall remain in effect, LICENSEE shall assume primary responsibility for enforcing the Patent Rights outside the Field of Use insofar as so doing is reasonably necessary or appropriate to protect LICENSEE's interest in respect of the Patent Rights as applicable to the Field of Use. The parties shall consult with each other regarding the legal and commercial significance of any infringement and the course of action to be taken. LICENSEE shall thereupon promptly contact alleged third party infringers of which LICENSEE is notified and take all commercially reasonable steps to persuade such third parties to desist from infringing the Patent Rights, which may include initiating and prosecuting an infringement action, or defending a challenge to the validity of the Patent Rights. Each party shall notify the other of each instance of alleged infringement of which it receives notice, and shall keep the other reasonably informed of all stages of Patent Rights enforcement. LICENSEE may use the name of SKI as party plaintiff. All costs and expenses including attorneys fees, of any action to enforce the Patent Rights taken by LICENSEE shall be borne by LICENSEE and LICENSEE shall keep any recovery of damages derived therefrom, the excess of such recovery over such costs and expenses shall be included in LICENSEE'S Net Sales. No settlement, consent judgment or other voluntary final disposition of the suit may be entered into without the



prior written consent of SKI, which consent shall not unreasonably be withheld, conditioned or delayed.

7.2 In the event LICENSEE becomes aware of unlicensed infringement of the Patent Rights either through notice from SKI or by other means, and does not, within six (6) months (a) secure cessation of the infringement; or (b) enter suit against the infringer; or (c) provide SKI with evidence of pendancy of a bona fide negotiation for sublicensing the infringer, then SKI, rather than and in lieu of LICENSEE'S performance under Section 7.1 above, shall have the right, upon thirty (30) days written notice to LICENSEE (and provided that LICENSEE still has not pursued (a), (b) or (c) above), to enforce the Patent Rights as against such identified infringement, and to sue for such infringement at SKI's own expense, and to collect for its own use any damages, profits and awards of whatever nature that it may recover for such infringement.

7.3 Each party shall promptly notify the other in writing in the event that a third party shall bring a claim of infringement against SKI or LICENSEE, either in the United States or in any foreign country in which there are Patent Rights.

7.4 In the event LICENSEE is sued for patent infringement, or enjoined from exercising its license rights granted hereunder, LICENSEE may elect to terminate this Agreement, in whole or in part as respects the area of alleged infringement, or contest the action against it. In any such action, LICENSEE shall be fully responsible for all its costs, including expenses, judgments and settlements, and shall be entitled to proceeds that it may recover, including judgments, settlements and awards, the excess of such recovery over such costs shall be included in LICENSEE'S Net Sales. .

7.5 In any infringement suit that either party may institute to enforce the Patent Rights against third parties pursuant to this Agreement, or in any infringement action brought against either party by a third party, each party hereto shall, at the request and expense of the other party, cooperate in all respects and, to the extent possible, have its employees testify when requested and make available relevant records, papers, information, samples, specimens, and the like.

#### ARTICLE VIII -INDEMNIFICATION, PRODUCT LIABILITY, REPRESENTATIONS

8.1 LICENSEE shall at all times during the term of this Agreement and thereafter, indemnify, defend and hold SKI and its Affiliates, their Board of Managers, officers, employees and affiliates (collectively, the "SKI Parties"), harmless against all claims and expenses, including legal expenses and reasonable attorneys' fees, arising out of the death of or injury to any person or persons or out of any damage to property and against any other claim, proceeding, demand, expense and liability of any kind whatsoever resulting from the production, manufacture, sale, use, lease, consumption or advertisement of the Licensed Product(s) or arising from any obligation of



LICENSEE hereunder, except to the extent, which the same shall be caused by the gross negligence or failure on the part of any SKI Party.

8.2 For the term of this Agreement, upon the commencement of clinical use, production, sale, or transfer, whichever occurs first, of any Licensed Product, insofar as available, LICENSEE shall obtain and carry in full force and effect general liability insurance which shall protect LICENSEE and SKI in regard to events covered by Section 8.1 above. Such insurance shall be written by a reputable insurance company, shall list SKI as an additional named insured thereunder, shall be endorsed to include liability coverage, and shall require thirty (30) days written notice to be given to SKI prior to any cancellation or material change thereof. The limits of such insurance shall not be less than two million dollars (\$2,000,000) per occurrence with an annual aggregate of five million dollars (\$5,000,000) for personal injury, death or property damage. LICENSEE shall provide SKI with Certificates of Insurance evidencing the same.

8.3 SKI represents that it holds title to its interest in the inventions under the Patent Rights with the exception of certain retained rights of the United States government, and that SKI has not licensed the Patent Rights to any third party. SKI further represents as of the Effective Date, to the best of its knowledge, that SKI is aware of no actions, suit or inquiry or investigation instituted by any federal or state governmental agency that questions the validity or purpose of this Agreement, or of any administrative or judicial proceedings contesting the inventorship, ownership, validity or enforceability of any element of the Patent Rights. SKI does not, however, warrant the validity of any patents, that any patents as yet unissued will issue, or the practice under such patents will be free of infringement.

8.4 Except as otherwise expressly set forth in this Agreement, SKI MAKES NO REPRESENTATIONS AND EXTENDS NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO, WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, AND VALIDITY OF PATENT RIGHTS CLAIMS, ISSUED OR PENDING, FREEDOM OF INFRINGEMENT OF ANY DOMESTIC OR FOREIGN PATENTS, COPYRIGHTS, TRADE SECRETS OR OTHER PROPRIETARY RIGHTS OF OTHER PARTIES. SKI IS LICENSING THE LICENSED PROPERTY ON AN "AS IS" BASIS. WITHOUT LIMITATION AS TO THE FOREGOING, NEITHER SKI, NOR ANY EMPLOYEE OR AGENT OF SKI, SHALL HAVE ANY LIABILITY TO LICENSEE, ITS SUBLICENSEES OR AFFILIATES, OR ANY OTHER PERSON ARISING OUT OF THE USE OF PATENTS, KNOW-HOW, PRODUCTS AND/OR ANYTHING DISCOVERED, DEVELOPED, MANUFACTURED, USED, SOLD, RENTED, LEASED OR OTHERWISE DISPOSED OF UNDER ANY LICENSE GRANTED HEREUNDER BY LICENSEE, ITS SUBLICENSEES OR AFFILIATES, OR ANY OTHER PARTY FOR ANY REASON, INCLUDING BUT NOT LIMITED TO, THE UNMERCHANTABILITY, INADEQUACY OR UNSUITABILITY OF THE PATENTS, TECHNICAL INFORMATION, PRODUCTS AND/OR ANYTHING DISCOVERED, DEVELOPED, MANUFACTURED, USED, SOLD, RENTED, LEASED OR OTHERWISE DISPOSED OF UNDER ANY LICENSE GRANTED HEREUNDER

FOR ANY PARTICULAR PURPOSE OR TO PRODUCE ANY PARTICULAR RESULT,  
OR FOR ANY LATENT DEFECTS THEREIN.

#### ARTICLE IX - EXPORT CONTROLS

It is understood that SKI is subject to United States Laws and regulations controlling the export of technical data, computer software, laboratory prototypes and other commodities (including the Arms Export Control Act, as amended and the Export Administration Act of 1979), and that its obligations hereunder are contingent on compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by LICENSEE that LICENSEE shall not export data or commodities to certain foreign countries without prior approval of such agency. SKI neither represents that a license shall not be required nor that, if required, it shall be issued.

#### ARTICLE X - NON-USE OF NAMES

LICENSEE shall not use the names of SKI or its Affiliates, nor any of their employees, nor any adaptation thereof, in any advertising, promotional or sales literature without prior written consent obtained from SKI in each case, provided, however, LICENSEE may use the name of SKI where the use of such name is required by law.

#### ARTICLE XI - ASSIGNMENT

11.1 This Agreement may not be assigned by LICENSEE without prior written consent from SKI, such consent not to be unreasonably withheld, conditioned or delayed.

11.2 Notwithstanding the foregoing prohibition, LICENSEE may without SKI's consent assign this Agreement to any entity that it may merge into, consolidate with, or transfer substantially all of its assets ("substantially" being Eighty Percent (80%) or more thereof) as an entirety, so long as the successor surviving corporation in any such merger, consolidation, transfer or reorganization assumes in writing the obligations of this Agreement. Such merger, consolidation, transfer or reorganization shall not in itself be a breach of this Article XI, nor be any default under this Agreement.

#### ARTICLE XII -TERM & TERMINATION

12.1 The term of the license granted under this Agreement shall commence on the Effective Date and shall continue, in each country, until the date of expiration of the last to expire patent included in the Patent Rights in that country, and if no patents issue, then for a term of twenty (20) years from the Effective Date.

12.2 SKI may terminate this Agreement if LICENSEE becomes insolvent or, a petition in bankruptcy is filed against LICENSEE and is consented to, acquiesced in or remains undismissed for sixty (60) days; or makes a general assignment for the benefit of creditors, or a receiver is appointed for LICENSEE, and LICENSEE does not return to solvency before the expiration of a sixty (60) day period.

12.3 Should LICENSEE fail to pay SKI license fees, royalties, milestones, patent expenses and any other payment due and payable hereunder for more than sixty (60) days after written notice, SKI shall thereupon have the right to terminate this Agreement on thirty (30) days written notice, unless LICENSEE shall pay SKI within the thirty (30) day period, all such license fees, royalties, milestones, patent expenses, and any other payments and interest due and payable. Upon the expiration of the thirty (30) day period, if LICENSEE shall not have paid all such license fees, royalties, milestones, patent expenses, and other payments and interest due and payable, the rights, privileges and license granted hereunder shall thereupon become terminable.

12.4 Upon any material breach of this Agreement by LICENSEE, other than those occurrences set out in Sections 12.2 and 12.3, hereinabove, which shall always take precedence in that order over any material breach or default referred to in this Section 12.4, SKI shall have the right to terminate this Agreement and the rights, privileges and license granted hereunder by ninety (90) days' notice to LICENSEE, provided that such breach shall not have been rectified prior to the expiration of such ninety (90) day period.

12.5 LICENSEE shall be entitled to terminate this Agreement in its entirety or as respects Field A or Field B, upon sixty (60) days written notice to SKI.

12.6 Upon termination of this Agreement, in whole or in part, for any reason, nothing herein shall be construed to release either party from any obligation that matured prior to the effective date of such termination. Insofar as this Agreement is terminated, LICENSEE must return to SKI all materials corresponding to Field A or Field B, or both, as applicable, relating to the Patent Rights and provided hereunder by SKI to LICENSEE and then continuing to remain in the possession of LICENSEE; provided, however, that, unless terminated under Sections 12.3 or 12.4, LICENSEE shall have the right for one year thereafter to dispose of all applicable Licensed Products then in its inventory, and shall pay royalties thereon, in accordance with the provisions of Article IV and shall submit the related reports as required by Article V, as though this Agreement had not terminated.

12.7 Other than any claim arising from LICENSEE'S failure to make payments due under this contract, any controversy or bona fide disputed claim arising between the parties to this Agreement, which dispute cannot be resolved by mutual agreement shall, by the election of either party, be resolved by submitting to dispute resolution before a fact-finding mediation body composed of one or more experts in the field, selected by mutual agreement within thirty (30) days of written request by either party. Said dispute resolution shall be held in New York, New York at such place as shall be mutually agreed upon in writing by the parties. The fact-finding body shall determine who shall bear the cost of said resolution. In the event that the parties cannot mutually agree within said thirty (30) days on the dispute resolution body, the parties will go to arbitration in accordance with the Commercial Arbitration Rules of the American Arbitration Association.

12.8 Upon termination of this Agreement as respects Field A or Field B, as applicable, for any reason all corresponding Sublicenses shall terminate, provided that, any corresponding Sublicensee shall have the opportunity to seek a license from SKI by advising SKI in writing within sixty (60) days after Sublicensee's receipt of notice of such termination.

12.9 Article VIII, Articles X, XIV and Section 12.6 of this Agreement shall survive termination, in whole or in part.

#### ARTICLE XIII -PAYMENTS, NOTICES AND OTHER COMMUNICATIONS

Any payment shall be made by remittance to SKI (Tax I.D. No. 13-1624 182) with "Payment, Contract No. SK#9927" indicated on the check, or as otherwise instructed by SKI. The check shall be sent to:

Memorial Sloan-Kettering Cancer Center  
Industrial Affairs  
P.O. Box 27718  
New York, NY 10087-27718

Any notice or other communication pursuant to this Agreement shall be sufficiently made or given when delivered by courier or other means providing proof of delivery to such party at its address below or as it shall designate by written notice given to the other party:

In the case of SKI:

Sloan-Kettering Institute for Cancer Research  
1275 York Avenue  
New York, New York 10021  
Attention: James S. Quirk  
Senior Vice President  
Research Resources Management

In the case of LICENSEE:

Errant Gene Therapeutics, LLC  
141 W. Jackson Boulevard  
Suite 300  
Chicago, Illinois 60604  
Attention: Patrick Girondi

#### ARTICLE XIV -CONFIDENTIALITY

14.1 The parties will exchange information which they consider to be confidential. Such information will include reports of LICENSEE'S development and commercialization plans and activities, and reports of NET SALES provided pursuant to this Agreement, and any other information which is marked as confidential at the time it is sent to the recipient. The recipient of confidential information of the other party agrees to employ all reasonable efforts to maintain the information confidential, such efforts to be no less than the degree of care employed by the recipient to preserve and safeguard its own confidential information. The information shall not be disclosed or revealed to anyone except employees, agents, investors or potential or actual collaborators of the recipient, or others, who have a need to know the information consistent with their position, and who have entered into a secrecy agreement with the recipient under which they are required to maintain confidential the proprietary information so disclosed to them, and shall have been advised by the recipient of the confidential nature of the information and that the information shall be treated accordingly. Notwithstanding the above, this Section shall not apply to disclosure of data or information to governmental or regulatory agencies.

14.2 The recipient's obligations under Paragraph 14.1 shall not extend to any part of the information:

- a. that can be demonstrated to have been in the public domain or publicly known and readily available to the trade or the public prior to the date of the disclosure; or
- b. that can be demonstrated, from written records to have been in the recipient's possession or readily available to the recipient

from another source not under obligation of secrecy to the disclosing party prior to the disclosure; or

- c. that becomes part of the public domain or publicly known by publication or otherwise, not due to any unauthorized act by the recipient; or
- d. that is demonstrated from written records to have been developed by or for the receiving party without reference to confidential information disclosed by the disclosing party; or
- e. that is required to be disclosed by law, government regulation or court order.

14.3 SKI shall be free to publish manuscripts, abstracts or the like relating to the work done at SKI related to the Patent Rights, pursuant to SKI's preserved right under Section 2.2 above;

#### ARTICLE XV - MISCELLANEOUS PROVISIONS

15.1 This Agreement shall be construed, governed, interpreted and applied in accordance with the laws of the State of New York, except that questions affecting the construction and effect of any patent shall be determined by the law of the country in which the patent was granted.

15.2 The provisions of this Agreement are severable, and in the event that any provisions of this Agreement shall be determined to be invalid or unenforceable under any controlling body of the law, such invalidity or unenforceability shall not in any way affect the validity or enforceability of the remaining provisions hereof.

15.3 LICENSEE agrees to mark the Licensed Products sold in the United States with all applicable United States patent numbers. All Licensed Products shipped to or sold in other countries shall be marked in such a manner as to conform with the patent laws and practice of the country of manufacture or sale.

15.4 If either party fails to fulfill its obligations hereunder when such failure is due to an act of God, or other circumstances beyond its reasonable control, including, without limitation to governmental or regulatory delay or inaction, fire, flood, civil commotion, riot, war (declared or undeclared), revolution or embargos, then said failure shall be excused for the duration of such event and for such time thereafter as is reasonable to enable the parties to resume performance under this Agreement, provided, however, that no event shall such time extend for a period of more than one hundred eighty (180) days.

15.5 The failure of either party to assert a right hereunder or to insist upon compliance with any term or condition of this Agreement shall not constitute a waiver of

that right or excuse a similar subsequent failure to perform any such term or condition by the other party.

15.6 This Agreement may be executed in any number of counterparts and each of such counterparts shall for all purposes be an original and all such counterparts shall together constitute but one and the same agreement.

IN WITNESS WHEREOF, authorized representatives of the parties have signed and dated this Agreement below.

Sloan-Kettering Institute for Cancer  
Research

Errant Gene Therapeutics, LLC

By: 

By: 

James S. Quirk  
Senior Vice President  
Research Resources Management

Patrick Girondi  
Chief Executive Officer

Date: 3/4/05

Date: 7 MAR 2005



*Senior Vice President  
Research Resources Management*

February 17, 2005

**TO WHOM IT MAY CONCERN:**

In my absence, Mr. Gustave J. Bernhardt, Director, Research Resources Management, will sign as an institutional official for the Sloan-Kettering Institute for Cancer Research.

James S. Quirk  
Senior Vice President

JSQ:em

*Memorial Sloan-Kettering Cancer Center  
1275 York Avenue, New York, New York 10021  
NCI designated Comprehensive Cancer Center*



**EXHIBIT A**

Patent Rights

- U.S. Patent Application No. 10/188,221 filed July 1, 2002. Vector Encoding Human Globin Gene and Use thereof in Treatment of Hemoglobinopathies based upon the following U.S. Provisional Applications: U.S. Provisional Application No. 60/301, 861 filed June 29, 2001; and U.S. Provisional Application No. 60/302, 852 filed July 2, 2001.
- International Application No. PCT/US02/20988.

EXHIBIT B

## Additional Data

A. Copies of the laboratory notebooks and documents which contain the details of the information (including without limitation, as applicable, preclinical data and results, toxicology studies, study or protocol design data and corresponding results) published in:

- a. the patent applications as identified on Exhibit A; and
- b. the following journal publications:

1. May, C., Rivella, S., Callegari, J., Hellers, G., Gaensler, K.M.L., Luzzatto, L., and Sadelain, M. 2000. Therapeutic haemoglobin synthesis in b-thalassaemic mice expressing lentivirus-encoded human b-globin. *Nature* 406:82-86.
2. May, C., Rivella, S., Chadburn, A., and Sadelain, M. 2002. Successful treatment of murine b-thalassemia intermedia by the transfer of the human b-globin gene. *Blood* 99:1902-1908.
3. Rivella, S. and Sadelain, M. 2002. Therapeutic globin gene delivery using lentiviral vectors. *Curr. Opin. Mol. Ther.* 4:505-514.
4. Rivella, S., May, C., Riviere, I., and Sadelain, M. 2003. A novel murine model of Cooley anemia and its rescue by lentiviral-mediated human b-globin gene transfer. *Blood* 101:2932-2938.
5. Sadelain, M., Wang, C.H.J., Antoniou, M., Grosveld, F., and Mulligan, R.C. 1995. Generation of a high-titer retroviral vector capable of expressing high levels of the human b-globin gene. *Proc. Natl. Acad. Sci. USA* 92:6728-6732.
6. Sadelain, M. 2004. Globin gene transfer as a potential treatment for the beta-thalassemias and sickle cell disease. *Vox Sang* 87:235-242.
7. Sadelain, M., Rivella, S., Isowski, L., Amakoglu, S., and Riviere, I. 2004. Globin gene transfer for treatment of the b-thalassemias and sickle cell disease. *Best Practice & Res. Clin. Haematol.* 17:517-534.

B. Copies of all preclinical data and results, toxicology studies, study or protocol design data and corresponding results obtained from the monkey studies now ongoing at SKI, whether now existing or arising at any time prior to the expiration of one year after the Effective Date, including copies of the related laboratory notebooks and of documents.

C. Instruction as to the current "art" applicable to the production of vector under the Patent Rights, insofar as readily available at the time of execution of the Agreement and able to be communicated by the Inventor with relatively minimal inconvenience.

Note: The data and information within the provisions of items A and B above may be used and disclosed, in accordance with article XIV of this agreement, for, among other purposes, the filing of an Investigational New Drug Application (IND) and New Drug Application (NDA) with the United States Food and Drug Administration.

# Exhibit B

## AGREEMENT

This Agreement is effective as of this 17<sup>th</sup> day of June, 2011 and is by and between Sloan-Kettering Institute for Cancer Research ("SKI") and New York membership corporation with principal offices at 1275 York Avenue, New York, New York 10065 and Errant Gene Therapeutics, LLC, a limited liability company formed under the laws of Delaware with principal offices located at 218 North Jefferson Street, Suite 300, Chicago, IL 60661, ("EGT").

Whereas, SKI and EGT are parties to a License Agreement effective March 07, 2005, identified as "Exclusive License Agreement for SKI's Technology 'SK972'" (License Agreement) under which EGT had certain rights and obligations with respect to the development and commercialization of a product for the treatment of Thalassemia and a product for treatment of Hemoglobinopathies other than Thalassemia (principally, but not limited to sickle-cell disorders) (the "Treatment");

Whereas, EGT has diligently worked since 2005 and expended substantial sums to develop the Treatment;

Whereas EGT is the owner of clinical grade vector TNS9.55.3 that was manufactured for use in a clinical trial for beta-thalassemia patients;

Whereas SKI wishes to acquire (a) the clinical grade vector TNS9.55.3 that was manufactured for use in a clinical trial for beta-thalassemia patients ; (b) information developed by EGT in connection with the Treatment (the "EGT Information"); and (c) EGT's US and EMEA orphan drug designations (the "Designations");

Whereas SKI licensed to EGT certain rights under the Patent Rights (defined in Section 1.10 of the License Agreement which is incorporated herein by reference), and

Whereas, SKI desires to acquire and assume all right to commercially develop the Treatment,, the Vector, the EGT Information and the Designations.

Now, therefore, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged:

1. The License Agreement is hereby terminated.

2. Definitions:

- a. "Vector" shall mean clinical grade vector TNS9.55.3 that was manufactured for use in a clinical trial for beta-thalassemia patients and any modification that was reasonably within contemplation of the parties at the time the vector was finalized as of September 30, 2010 for the purpose of using the vector TNS9.3.55 for the treatment of sickle cell disorder as opposed to thalassemia.

b. "Modified Vector" shall mean the Vector and any modification or improvement thereof but only to the extent that such modification or improvement is such that the FDA would not require a new IND to be filed over the IND for the TNS9.3.55 vector as it exists at the time of this agreement.

3. EGT hereby sells, assigns and transfers to SKI all of EGT's right, title and interest in and to, the Vector and EGT Information.

4. SKI will continue with its work to promptly file an investigational new drug application (IND) for Vector and diligently proceed with clinical trials on three patients and use its best efforts to treat at least three (3) patients under the current trial in accordance with the IND protocol and in accordance with the data reporting requirements of the subject FDA protocol. SKI will provide to EGT trial information, as permitted by law.

5. SKI agrees that it will use its best efforts to treat at least three (3) patients under the current trial in accordance with the IND protocol and in accordance with the data reporting requirements of the subject FDA protocol. Per the requirements of License Agreement Article 3.1, SKI will also use its best efforts to reach a medically reasonable determination as to the efficacy of the vector, based on the data collected from the first three patients. Nothing herein shall be construed to obligate SKI to develop any therapy for thalassemia or sickle cell anemia which, in its sole medical judgment is not the best potential treatment for patents.

6. SKI shall pay to EGT fifty percent (50%) of all consideration received directly or indirectly by SKI or any affiliate or subsidiary of SKI, from any license, sale or commercial exploitation of any Treatment using the Vector or Modified Vector throughout the world, including but not limited to license fees, advances, milestones, royalty payments, and other payment mechanisms, and income from the commercial exploitation of any therapy employing the Vector or the Modified Vector. Such payment shall continue for as long as such consideration is received by SKI.

7. SKI shall provide to EGT a complete copy of any license agreements entered into by SKI with respect to the Vector or Modified Vector.

8. All payments shall be made to EGT within sixty (60) business days of the receipt of the consideration by SKI. SKI shall pay to EGT interest on any payments not paid when due equal to two percent above the daily prime rate of interest announced from time to time by JP Morgan Chase Bank. All payments shall be accompanied by a statement showing all consideration received by SKI and giving the particulars of all such business related to such consideration.

9. SKI shall keep full, true and accurate books of account containing all particulars that may be necessary for the purpose of showing the amounts payable to EGT hereunder. Said books and records shall be maintained for a period of no less than five (5) years following the period to which they pertain. For the term of this agreement, upon

reasonable written notice, SKI shall allow EGT or its agents to inspect such books and records for the purpose of verifying SKI's payments.

10. For good and valuable consideration the receipt and sufficiency of which is hereby acknowledged, SKI hereby releases and discharges EGT, its representatives, officers, directors, shareholders, employees, attorneys, agents, principals, subsidiaries, affiliate corporations, and successors and assigns from all actions, causes of action, suits, debts, dues, sums of money, accounts, reckonings, bonds, bills, controversies, agreements, damages, judgments, executions, claims and demands whatsoever, known or unknown in law or equity from the beginning of time until the date hereof, that SKI ever had, now has, or hereafter can, shall, or may have, including but not limited to any liability arising out of the use of the Vector or Modified Vector in any SKI clinical trial and, any litigation or claims of damages arising out of the subsequent development of the Vector or Modified Vector or the licensing the rights to such vector by SKI, except that that there shall be no release related to any duties and obligations imposed by this Agreement

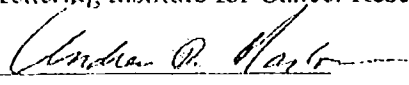
11. For good and valuable consideration the receipt and sufficiency of which is hereby acknowledged, EGT hereby releases and discharges SKI, its representatives, officers, directors, shareholders, employees, attorneys, agents, principals, subsidiaries, affiliate corporations, and successors and assigns from all actions, causes of action, suits, debts, dues, sums of money, accounts, reckonings, bonds, bills, controversies, agreements, damages, judgments, executions, claims and demands whatsoever, known or unknown in law or equity from the beginning of time until the date hereof, that EGT ever had, now has, or hereafter can, shall, or may have, except that there shall be no release related to any duties and obligations imposed by this Agreement.

12. Each of the parties shall cooperate in the preparation and execution of such additional documents as are required to carry out the terms of this Agreement.

13. This agreement may be executed in counterparts, each of which shall be deemed an original but all of which shall constitute one instrument. Facsimiles shall be deemed originals.

IN WITNESS WHEREOF, the undersigned have executed this Agreement as of the date written above.

Sloan-Kettering Institute for Cancer Research

By: 

Date: 6/17/11

Errant Gene Therapeutics, LLC

By: \_\_\_\_\_

Date: \_\_\_\_\_

reasonable written notice, SKI shall allow EGT or its agents to inspect such books and records for the purpose of verifying SKI's payments.

10. For good and valuable consideration the receipt and sufficiency of which is hereby acknowledged, SKI hereby releases and discharges EGT, its representatives, officers, directors, shareholders, employees, attorneys, agents, principals, subsidiaries, affiliate corporations, and successors and assigns from all actions, causes of action, suits, debts, dues, sums of money, accounts, reckonings, bonds, bills, controversies, agreements, damages, judgments, executions, claims and demands whatsoever, known or unknown in law or equity from the beginning of time until the date hereof, that SKI ever had, now has, or hereafter can, shall, or may have, including but not limited to any liability arising out of the use of the Vector or Modified Vector in any SKI clinical trial and, any litigation or claims of damages arising out of the subsequent development of the Vector or Modified Vector or the licensing the rights to such vector by SKI, except that that there shall be no release related to any duties and obligations imposed by this Agreement

11. For good and valuable consideration the receipt and sufficiency of which is hereby acknowledged, EGT hereby releases and discharges SKI, its representatives, officers, directors, shareholders, employees, attorneys, agents, principals, subsidiaries, affiliate corporations, and successors and assigns from all actions, causes of action, suits, debts, dues, sums of money, accounts, reckonings, bonds, bills, controversies, agreements, damages, judgments, executions, claims and demands whatsoever, known or unknown in law or equity from the beginning of time until the date hereof, that EGT ever had, now has, or hereafter can, shall, or may have, except that there shall be no release related to any duties and obligations imposed by this Agreement.

12. Each of the parties shall cooperate in the preparation and execution of such additional documents as are required to carry out the terms of this Agreement.

13. This agreement may be executed in counterparts, each of which shall be deemed an original but all of which shall constitute one instrument. Facsimiles shall be deemed originals.

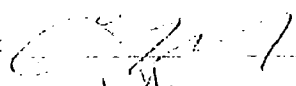
IN WITNESS WHEREOF, the undersigned have executed this Agreement as of the date written above.

Sloan-Kettering Institute for Cancer Research

By: \_\_\_\_\_

Date: \_\_\_\_\_

Errant Gene Therapeutics, LLC

By:  \_\_\_\_\_

Date:  \_\_\_\_\_



# Exhibit C

**CONFIDENTIAL SETTLEMENT AGREEMENT**

This Confidential Settlement Agreement, dated as of November 2, 2020, sets forth the agreement by and between Errant Gene Therapeutics, LLC and Patrick Girondi (collectively "EGT"), Memorial Sloan Kettering Cancer Center and The Sloan Kettering Institute for Cancer Research (collectively "MSK") and bluebird bio, Inc. ("BBB") (EGT, MSK, and BBB, collectively, the "Parties"), for good and valuable consideration, to resolve any and all disputes among them, including, without limitation, any and all disputes related to the 2005 Agreement, the 2011 Agreement, the New York Litigation and the Massachusetts Litigation (collectively the "Dispute").

WHEREAS, MSK and EGT entered into a March 7, 2005 Exclusive License Agreement ("2005 Agreement") pursuant to which MSK granted EGT an exclusive license to certain patents and patent applications; and

WHEREAS, MSK and EGT entered into a June 17, 2011 Agreement ("2011 Agreement") as amended that terminated the 2005 Agreement, and pursuant to which EGT sold, assigned and transferred to MSK all of EGT's right, title and interest in the clinical grade vector TNS9.3.55 and related information; and

WHEREAS, EGT filed a lawsuit against MSK and BBB in the Supreme Court of the State of New York captioned *Errant Gene Therapeutics, LLC v. Sloan Kettering Institute for Cancer Research and Bluebird Bio Inc.*, Index No. 150856/2017 (the "New York Litigation"); and

WHEREAS, EGT filed a lawsuit against Third Rock Ventures, LLC and Nick Leschly (former partner at Third Rock and CEO of BBB) in the Superior Court in the Commonwealth of Massachusetts captioned *Errant Gene Therapeutics, LLC v. Third Rock Ventures, LLC and Nick Leschly*, Civil Action No. 19-1832 (the "Massachusetts Litigation"); and

WHEREAS, the Parties have now agreed to resolve the Dispute in order to avoid further disputes and the risks of further litigation, and contain associated fees, costs and expenses thereof.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and other good and valuable consideration including the payment of the Settlement Amount to EGT as specified herein, the Parties, intending to be legally bound hereby, agree to the following terms.

1. [REDACTED] **Terms.** To resolve any and all disputes between the Parties related to the Dispute [REDACTED]

①



2. Return of Vector. To resolve any and all disputes between the Parties related to the Dispute, MSK shall:

- a. Give EGT an [REDACTED] to the intellectual property licensed in the [REDACTED];
- b. Sell, assign, and transfer to EGT the following: (i) all of MSK's right, title and interest in and to the [REDACTED]; (ii) any and all information and improvements developed by EGT or MSK (but only to the extent that the information and improvements were developed on or before June 17, 2011) in connection with the development and commercialization of a product for the treatment of Thalassemia and a product for treatment of Hemoglobinopathies other than Thalassemia (principally, but not limited sickle-cell disorders) (the "Treatment"); and (iii) regulatory submissions, clinical trial data, and communications concerning the [REDACTED] vector or the Treatment;
- c. Cooperate with EGT and its agents with regard to the transfer of information in possession of the trial's principal investigator with respect to the clinical trial for the Vector and/or Treatment;
- d. Return any and all remaining portions of the clinical grade [REDACTED], along with any and all information concerning the biobanking, testing, and/or analysis of the vector within sixty (60) days of the execution of this Agreement; and
- a. MSK agrees to give give EGT [REDACTED]  
[REDACTED] and for which developing making, having made, using, importing, selling or offering to sell the [REDACTED] vector or any modified or related lentiviral vector would be or could be infringed.

For the avoidance of doubt, nothing herein shall prevent MSK from conducting internal research using non-clinical grade [REDACTED].



EGT and MSK agree to that to the extent they need to take any further action or execute any other documents to effectuate the transfer of rights set forth in this Paragraph 2, they will work cooperatively to do so.

3. Dismissal of Litigation. EGT will dismiss with prejudice the New York Litigation and the Massachusetts Litigation within (3) business days of the execution of this Agreement.
4. Prior Agreements Terminated. The 2005 Agreement (to the extent effective) and the 2011 Agreement as amended are hereby terminated, and the Parties' rights and obligations shall be determined exclusively with reference to this Confidential Settlement Agreement.
5. Mutual Releases. The Parties exchange mutual general releases which shall include, without limitation, all claims, both at law and in equity, accrued or unaccrued, known or unknown, suspected or unsuspected that relate to or arise out of (i) the 2005 Agreement, (ii) the 2011 Agreement, (iii) the Dispute, (iv) the actions of any of the Parties or any Affiliate of any Party leading to the execution of the 2005 Agreement or the 2011 Agreement, including any costs, expenses and legal fees. For the avoidance of doubt, the mutual general releases shall release all claims, both at law and in equity, accrued or unaccrued, know or unknown, suspected or unsuspected that were brought or could have been brought by EGT in the New York Litigation and the Massachusetts Litigation.

EGT, and each of its predecessors, successors, assigns, officers, directors, employees, trustees, attorneys, parents, subsidiaries and affiliates (collectively, "**EGT Releasors**") fully, finally and forever release, relinquish, acquit and discharge MSK and all of MSK's past and present partners and associates, principals, shareholders, members, directors, officers, representatives, predecessors, successors, partnerships, corporations, heirs, executors, administrators, assigns, insurers, reinsurers, employees and attorneys (collectively the "**MSK Releasees**") from and against any and all claims, causes of action, demands, disputes, suits, debts, dues, liabilities, sums of money, accounts, reckonings, specialties, bonds, covenants, contracts, agreements, controversies, promises, assessments, rights, damages, costs and/or expenses whether based on a tort, contract or any other theory of recovery, in law, admiralty or equity, whether known or unknown, suspected or unsuspected, asserted or unasserted, foreseen or unforeseen, that EGT Releasors may have, ever had or now has against the MSK Releasees or any of them, for upon or by reason of any cause or thing, from the beginning of the world to the Parties' execution of this Confidential Settlement Agreement.

EGT Releasors fully, finally and forever release, relinquish, acquit and discharge BBB and all of BBB's past and present partners and associates, principals, shareholders, members, directors, officers, representatives, predecessors, successors, partnerships, corporations, heirs, executors, administrators, assigns, insurers, reinsurers, employees and attorneys (collectively the "**BBB Releasees**") from and against any and all claims, causes of action, demands, disputes, suits, debts, dues, liabilities, sums of money, accounts, reckonings, specialties, bonds, covenants, contracts, agreements, controversies, promises, assessments, rights, damages, costs and/or expenses whether based on a tort, contract or

any other theory of recovery, in law, admiralty or equity, whether known or unknown, suspected or unsuspected, asserted or unasserted, foreseen or unforeseen, that EGT Releasors may have, ever had or now has against the BBB Releasees or any of them, for upon or by reason of any cause or thing, from the beginning of the world to the Parties' execution of this Confidential Settlement Agreement.

MSK, and each of its predecessors, successors, assigns, officers, directors, employees, trustees, attorneys, parents, subsidiaries and affiliates (collectively, "**MSK Releasors**") fully, finally and forever release, relinquish, acquit and discharge EGT and all of EGT's past and present partners and associates, principals, shareholders, members, directors, officers, representatives, predecessors, successors, partnerships, corporations, heirs, executors, administrators, assigns, insurers, reinsurers, employees and attorneys (collectively the "**EGT Releasees**") from and against any and all claims, causes of action, demands, disputes, suits, debts, dues, liabilities, sums of money, accounts, reckonings, specialties, bonds, covenants, contracts, agreements, controversies, promises, assessments, rights, damages, costs and/or expenses whether based on a tort, contract or any other theory of recovery, in law, admiralty or equity, whether known or unknown, suspected or unsuspected, asserted or unasserted, foreseen or unforeseen, that MSK Releasors may have, ever had or now has against the EGT Releasees or any of them, for upon or by reason of any cause or thing, from the beginning of the world to the Parties' execution of this Confidential Settlement Agreement.

MSK Releasors fully, finally and forever release, relinquish, acquit and discharge BBB's Releasees from and against any and all claims, causes of action, demands, disputes, suits, debts, dues, liabilities, sums of money, accounts, reckonings, specialties, bonds, covenants, contracts, agreements, controversies, promises, assessments, rights, damages, costs and/or expenses whether based on a tort, contract or any other theory of recovery, in law, admiralty or equity, whether known or unknown, suspected or unsuspected, asserted or unasserted, foreseen or unforeseen, that MSK Releasors may have, ever had or now has against the BBB Releasees or any of them, for upon or by reason of any cause or thing, from the beginning of the world to the Parties' execution of this Confidential Settlement Agreement.

BBB and each of its predecessors, successors, assigns, officers, directors, employees, trustees, attorneys, parents, subsidiaries and affiliates (collectively, "**BBB Releasors**") will irrevocably, unconditionally and forever release and discharge the EGT Releasees from and against any and all claims, causes of action, demands, disputes, suits, debts, dues, liabilities, sums of money, accounts, reckonings, specialties, bonds, covenants, contracts, agreements, controversies, promises, assessments, rights, damages, costs and/or expenses whether based on a tort, contract or any other theory of recovery, in law, admiralty or equity, whether known or unknown, suspected or unsuspected, asserted or unasserted, foreseen or unforeseen, that BBB Releasors may have, ever had or now has against the EGT Releasees or any of them, for upon or by reason of any cause or thing, from the beginning of the world to the Parties' execution of this Confidential Settlement Agreement.



BBB Releasors fully, finally and forever release, relinquish, acquit and discharge MSK Releasees from and against any and all claims, causes of action, demands, disputes, suits, debts, dues, liabilities, sums of money, accounts, reckonings, specialties, bonds, covenants, contracts, agreements, controversies, promises, assessments, rights, damages, costs and/or expenses whether based on a tort, contract or any other theory of recovery, in law, admiralty or equity, whether known or unknown, suspected or unsuspected, asserted or unasserted, foreseen or unforeseen, that BBB Releasors may have, ever had or now has against the MSK Releasees or any of them, for upon or by reason of any cause or thing, from the beginning of the world to the Parties' execution of this Confidential Settlement Agreement.

Each Party waives to the fullest extent permitted by law the provisions and benefits of Section 1542 of the California Civil Code, which provides that: A GENERAL RELEASE DOES NOT EXTEND TO CLAIMS THAT THE CREDITOR OR RELEASING PARTY DOES NOT KNOW OR SUSPECT TO EXIST IN HIS OR HER FAVOR AT THE TIME OF EXECUTING THE RELEASE AND THAT, IF KNOWN BY HIM OR HER, WOULD HAVE MATERIALLY AFFECTED HIS OR HER SETTLEMENT WITH THE DEBTOR OR RELEASED PARTY.

6. Applicable Law. This Confidential Settlement Agreement and all disputes arising hereunder or relating hereto shall be governed by the laws of the State of New York without giving effect to the conflict of laws principles thereof.
7. Dispute Resolution. The Parties shall make all reasonable efforts to resolve any dispute concerning this Confidential Settlement Agreement, its construction, or its actual or alleged breach, by face-to-face negotiations between senior executives. Should such negotiation fail to resolve the matter, as defined by either party sending written notice to the other party of an impasse after at least one face-to-face negotiation meeting among senior executives of the parties, the matter shall be finally decided by David Ichel of X-Dispute, LLC or, if Mr. Ichel is not available, three (3) neutral arbitrators (the "Tribunal") seated in New York, New York under the AAA Arbitration Rules (the "Rules").
8. Representations. Each of the Parties represents and warrants that it has full power and authority to enter into this Confidential Settlement Agreement, has consulted legal counsel, understands the terms hereof, enters into this Confidential Settlement Agreement as of its own free will, and is not relying on any promise, understanding, or representation of any other party, except as set forth herein, and that the person signing on behalf of each of the Parties is duly authorized to sign on behalf of such party.
9. Execution. This Confidential Settlement Agreement may be executed in counterparts, including by signature transmitted by email. Each counterpart when so executed shall be deemed to be an original, and all such counterparts together shall constitute the same instrument.

10. Entire Agreement. This Confidential Settlement Agreement sets forth the entire agreement and understanding between the Parties and supersedes and cancels all previous negotiations, agreements and commitments, whether oral or in writing, with respect to the subject matter hereof. No deletion, amendment or addition to this Confidential Settlement Agreement shall be effective unless in writing signed by all Parties.
11. Confidentiality. The Parties agree that the existence and terms of this Confidential Settlement Agreement is strictly confidential and shall not be disclosed unless otherwise required by law. The Parties agree that, except to enforce this Confidential Settlement Agreement, make a disclosure to legal counsel, insurers, and/or accountants, or unless agreed to by the Parties in writing or required by law, the Parties, their respective parents, affiliates, successors, assigns, and their employees, officers, directors and other representatives shall not publish or otherwise disclose the contents of this Confidential Settlement Agreement. No Party may disclose this Confidential Settlement Agreement or the confidential terms herein without first obtaining the written approval of the non-disclosing Party(ies), except as required by law. If a Party believes that the disclosure of all or portions of this Confidential Settlement Agreement is required by applicable law, then that Party shall inform the other Party in writing ten business days prior to any such disclosure to allow the other Party(ies) sufficient time prior to any such disclosure to allow the other Party to seek a protective order prior to any such disclosure.

The Parties Agree that the only statement they will make about the Confidential Settlement Agreement is the following: "The parties have reached a mutually agreeable resolution in the best interests of the patients."



Executed as of this 2nd day of November, 2020.

**Errant Gene Therapeutics, LLC**

BY:  \_\_\_\_\_

NAME:

TITLE:

**Patrick Gironidi**

BY:  \_\_\_\_\_

NAME:

TITLE:

**Memorial Sloan Kettering Cancer Center  
and The Sloan Kettering Institute**

BY: \_\_\_\_\_

NAME:

TITLE:

**bluebird bio, Inc.**

BY: \_\_\_\_\_

NAME:

TITLE:



Executed as of this 2nd day of November, 2020.

**Errant Gene Therapeutics, LLC**

BY: \_\_\_\_\_

NAME:

TITLE:

**Patrick Gironidi**

BY: \_\_\_\_\_

NAME:

TITLE:

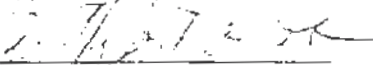
**Memorial Sloan Kettering Cancer Center  
and The Sloan Kettering Institute**

BY: 

NAME: Eric M. Cottingham

TITLE: Senior VP, Research &  
Technology Management

**bluebird bio, Inc.**

BY: 

NAME: Timothy J. Douros

TITLE: SVP, General Counsel

# Exhibit D

**SUPREME COURT OF THE STATE OF NEW YORK  
COUNTY OF NEW YORK**

ERRANT GENE THERAPEUTICS, LLC,

Plaintiff,

Index No. 150856/2017

v.-

**SECOND AMENDED  
COMPLAINT**

SLOAN-KETTERING INSTITUTE FOR CANCER  
RESEARCH and BLUEBIRD BIO INC.,

Defendants.

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Plaintiff Errant Gene Therapeutics LLC ("EGT"), by its attorneys, McCue Sussmane Zapfel Cohen & Youbi, P.C., for its Second Amended Complaint against Sloan-Kettering Institute for Cancer Research ("SKI" or "MSKCC") and Bluebird Bio Inc. ("Bluebird" or "BBB") herein alleges as follows:

**NATURE OF THE CASE**

1. Thalassemia is a fatal inherited blood disorder in which mutated genes cause the production of abnormally low levels of hemoglobin and red blood cells. The average age of mortality for patients with severe forms of the disease is reported as twenty-eight years.
2. Sick cell disease is a group of inherited blood disorders characterized by chronic anemia, painful events, and various complications due to associated tissue and organ damage. In the United States, sickle cell disease is most often seen in African Americans.
3. This action states causes of action for breach of contract, fraud, conspiracy to defraud, unfair competition and unjust enrichment in connection with a potentially life-saving gene therapy cure for these diseases developed by EGT (the "EGT Vector"), which was intentionally abandoned by SKI solely for Bluebird's benefit.

## PARTIES

4. Plaintiff EGT is a biopharmaceutical company dedicated to the development of treatments for underserved, rare, life-threatening diseases, commonly referred to as "orphan diseases." EGT is a Delaware limited liability company whose place of business is located at 218 North Jefferson Street, Suite 300, Chicago, Illinois 60661. Patrick Girondi is the Chief Executive Officer of EGT.

5. Defendant SKI is a New York membership 'not for profit' cancer research institute and hospital with annual revenues of over \$2.2 billion, with principal offices at 1275 York Avenue, New York, New York 10021.

6. Defendant Bluebird is a Delaware corporation having its headquarters at 150 Second Street, Cambridge, Massachusetts.

## JURISDICTION & VENUE

7. The jurisdiction of this Court is proper pursuant to CPLR §§ 301 and 302.

8. Venue is proper pursuant to CPLR § 503.

## STATEMENT OF FACTS

### EGT's Development of the Technology

9. In October 1992, Patrick Girondi's eldest son, Rocco Girondi, was diagnosed with Thalassemia, a fatal blood disease. In 1993, Patrick Girondi left a successful trading career and founded a pharmaceutical company with hopes of curing that disease.

10. Since 1993, EGT and its predecessor have built a grass roots organization including and not limited to five Thalassemia organizations, in Asia, Europe and North America and a Sickle Cell Disease organization in the United States. Each of these organizations have supported and closely monitored all steps of the development of the EGT Vector. That represents over twenty

years of work by committed executives, scientists and investments exceeding \$35,000,000 from private donors, investors, and grants.

11. EGT developed the EGT Vector as the result of relationships with world-wide research centers, including but not limited to Boston University, St. Jude Children's Research Hospital, University of Milano, Gene and Cell Therapy Center in Thessaloniki, Greece, Nasik Hospital, in Nasik India and Children's Hospital Oakland Research Institute.

12. In 2000, after seven years of personally sponsoring different research projects, including conducting a clinical trial for thirty-eight (38) patients, Patrick Girondi began supporting SKI researchers Stefano Rivella ("Rivella") and Michel Sadelain ("Sadelain") in their roles in the invention of a technology that had cured five generations of Thalassemic mice.

13. In 2003, Patrick Girondi was informed by Sadelain that SKI would no longer support their gene therapy project. SKI is expressly an institute for cancer research. SKI was not interested in gene therapy for blood disorders. This was confirmed to Patrick Girondi by Sadelain and Dr. Lucio Luzzatto ("Luzzatto"), the person who was involved in hiring Sadelain and Rivella at SKI. Luzzatto was also the president of the ethics committee for gene therapy at the FDA for three years.

14. Dr. Norbert Wiech, a distinguished scientist and colleague to the then SKI President, today Director of the NIH, Harold Varmus ("Varmus"), and Patrick Girondi attempted to interest pharmaceutical companies in the project. Those companies all said the same thing, 'gene therapy' was premature and though many sympathized with the mission of a father fighting to save a son, from an investment point of view, the project was frivolous.

15. In March, 2005, with no other parties interested in saving the abandoned project, SKI sold the exclusive worldwide rights to exploit the potential gene therapy treatment which became the EGT Vector for use in blood disorders known as hemoglobinopathies, including

Thalassemia, (also known as Cooley's Anemia), and Sickle Cell Disease. A cure for patients with Thalassemia and Sickle Cell Diseases became the sole goal of EGT. A copy of the agreement (the "2005 Agreement") is attached hereto as Exhibit A.

16. At the time of the 2005 Agreement, no known human clinical data existed to support the viability of gene therapies for Thalassemia and Sickle Cell Disease.

17. SKI was unwilling to fund and unable to secure outside funding for the research, and without the transfer to EGT, the research would have died.

18. Thalassemia and Sickle Cell Disease (Anemia) patients must hyper transfuse blood and receive painful iron chelation medications to reduce iron build-up that comes with the blood transfusions. The only established cure is a bone marrow or stem cell transplant. Less than 25% of patients have compatible donors.

19. Upon entering into the 2005 Agreement, EGT immediately began implementing the research, funding at least three separate, scientific contracts with SKI and engaging international specialists and researchers. This culminated into over four years of research and the development of the EGT Vector.

#### **EGT Performed All Obligations Under The 2005 Agreement**

20. EGT's network dedicated to developing and improving the Vector grew to include major research institutions and hospitals, such as, AMC Amsterdam, Bambino Gesù, Fairview Institute Minneapolis, Gene Center Cagliari, NIH, Notre Dame University, Policlinico Milano, Ospedale Cervello Palermo, San Matteo Pavia, University Mainz Germany and the State University of New York at Albany.

21. EGT collaborated with such companies as Sigma Tau, Voisin in Switzerland, Biomarin, Aldevron and Baxter.

22. EGT is affiliated with or has received support from Cooley's Anemia Foundation, Cooley's Anemia International, Giambrone Foundation, Italian Telethon, Jackie Robinson Foundation, Thalassemia International Federation, Thalassemia Foundation India, The Think Foundation of Mumbai, India and the World Health Organization.

23. Prior to and at all times since the date of the 2005 Agreement, EGT has used commercially reasonable efforts to develop and introduce to the markets a gene therapy treatment for Thalassemia and Sickle Cell Diseases through a thorough and diligent program consistent with sound and reasonable scientific and business judgment.

24. EGT committed every available resource to develop its technology into a gene therapy treatment known and trademarked by EGT as "Thalagen."

25. EGT welcomed SKI participation in major decisions, such as the timing of applications for regulatory approval to clinically treat patients.

26. EGT followed a comprehensive business, research and development plan and schedule, keeping SKI abreast of every detail.

27. EGT continued the grass roots effort to support the Thalassemia and Sickle Cell Disease research, involving hundreds of patients and patient groups throughout the world, many of whom are anxiously awaiting clinical trials to continue for Thalagen. EGT won awards and recognition from, among others, the Indian Thalassemia Foundation and the World Health Organization.

28. EGT diligently followed the process of obtaining U.S. governmental approval for advancing the therapy.

29. In 2007, EGT obtained Orphan Drug Designation in the United States which provides the designee with a period of market exclusivity for an approved drug.

30. In 2007, EGT obtained the unanimous approval of the NIH Recombinant DNA Advisory Committee (RAC). RAC is comprised of renowned experts and is a necessary hurdle for all gene therapies. EGT submitted a coherent file, composed of thousands of pages, based on demonstrating patient safety.

31. In 2008, EGT completed the pre-IND (Investigative New Drug) meeting with the FDA and trademarked the name “Thalagen” for its therapy for Sickle Cell Disorder and Thalassemia therapy. In 2009, EGT was awarded Orphan Drug Designation in Europe.

32. On June 2, 2009, a US Patent was awarded to the inventors of the Vector, including Sadelain and Rivella, the rights to which were exclusively licensed to EGT under the 2005 Agreement. This is the basis for Thalagen and is the intellectual property that was developed by EGT under the 2005 Agreement.

#### **Production of Vector by EGT Using Highest Standards**

33. EGT took the additional time necessary to produce Vector in accordance with the highest standard of manufacturing and testing, which is designated by the FDA as chemical Good Manufacturing Practice (cGMP).

34. EGT developed the Vector designated for human use by employing a highly purifying filtration regimen and meeting U.S standards for safety, stability, purification and potency. The manufacturer must be accredited by the FDA and each cGMP production batch of Vector requires filings with the FDA. Such filing is referred to as the Drug Master File.



35. To achieve the patient safety-centric standard, four years of testing and refinement were necessary. The Vector had not been ready for Clinical Trials as SKI had initially claimed when they sold the technology to EGT in 2005.

36. On September 1, 2010, EGT completed physical production of the medicine for Phase I of the clinical trial. It took eighteen (18) months and over \$1.3 million funded by EGT. Gene therapy was now at the forefront of the pharmaceutical industry and backed by billions of dollars of research. EGT has leapt over various hurdles to develop a tremendously promising medicine, leading the competitive and lucrative race for the first successful gene therapy treatment.

37. EGT developed and produced a Vector that is closer to the endogenous (natural) gene than the unfiltered and potentially dangerous vector used by EGT's competitor Genetix, in French clinical trials outside the supervision of the FDA. Today Genetix is known as Bluebird Bio Inc. ("Bluebird").

38. The FDA was presented with a previously untested and novel method of collecting patient bone marrow stem cells. This method reduces risk to the patient and enables optimal engraftment. EGT's competitor, Bluebird, relied on a less patient-friendly procedure.

39. SKI requested that EGT delay clinical trials of Thalagen until the confirmation of the new method of collecting stem cells.

40. SKI insisted that the study was necessary to prove efficacy of the method of harvesting bone marrow stem cells. In the study, a patient's bone marrow was stimulated and then stem cells were collected from the blood.

41. An FDA pilot study of the mobilization method was conducted in the US and Greece from 2008 through October 2010.

42. EGT could not apply for FDA approval for its clinical trials until after the study had proven that the new method of collecting stem cells from blood in thalassemia patients was effective.

43. EGT assisted Italian centers to arrange for Thalassemic patients to travel to New York to participate in the mobilization study (without any immediate treatment for their disease).

44. EGT successfully spearheaded the application for a \$500,000 grant from the Italian Telethon for the stem cell mobilization trial.

45. From 2006 to 2009, EGT paid to SKI over \$555,000 to conduct additional laboratory work to modify the EGT technology to improve efficacy in human patients and which was necessary for commercial development.

46. EGT paid to SKI scientists consulting fees of \$204,000 to perform critical laboratory experimentation for EGT.

**SKI and Bluebird Enter into A Secret Partnership to Wrest the EGT Vector  
and EGT Intellectual Property from EGT**

47. In 2009, the French government and the FDA halted gene therapy clinical trials in France and the US because of improper dominance of certain cell growth during a clinical trial performed on thalassemic patients by Genetix Pharma.

48. In March 2010, Third Rock Ventures LLC (“Third Rock”) bought Genetix for \$35,000,000 and changed its name to Bluebird to distance itself from the 2009 ban on gene therapy.

49. In March 2010, with only a vector that was deemed so unsafe that all gene therapy was banned, Third Rock’s first step was to approach SKI to purchase the EGT Vector. Such purchase would have eliminated Bluebird’s competition which was poised to start clinical trials and would have provided Bluebird with the intellectual property necessary to make a safe vector.

50. In an internal email, SKI's Associate Director of the Office of Industrial Affairs warned about the impending doom which would result from dealing with Bluebird:

They (Bluebird) know we entered into a licensee with EGT, and they are coming to you, not EGT when they know perfectly well that they should talk to EGT to get the rights to the technology." She further advised that "I don't see how 3<sup>rd</sup> Rock Genetix can make the best out of MSK/Genetix technology without letting one **sit on the shelf.**" (Emphasis added)

51. Third Rock and Bluebird executives met with SKI. Third Rock wanted to purchase the EGT Vector, claiming that it was superior to the Bluebird vector which they had just purchased in a \$35 million deal from Sadelain's former Harvard classmate.

52. In a June 2010 email, SKI warned EGT about Bluebird's motives in arranging a meeting with EGT. It stated:

The stakes are very high now and in the next few weeks. **You can count on GP (now Bluebird) to proactively sabotage all your efforts (bold in original)** . . . We've just spent 2 years improving the manufacturing. We made enough vector for 10 patients in one production run. GP makes one batch at the time for one patient. That is not viable. Their vector has an unstable structure ("it rearranges", as found in the second patient). That makes it very unlikely that it will ever be commercialized, at least with its current sequence. Based on published mouse studies, our vector expresses better than theirs.

The [EGT] protocol

- A. Is approved in the US, and same will likely be approved in Italy and Greece.
- B. We have carefully prepared it, including, amongst other things, collecting patient cells (all "mobilizations" were very safe and successful).
- C. Is endorsed by many thal docs and patients, who agree that starting with myeloablation is too aggressive.
- D. We have orphan drug status to develop it.

3. The team

- A. We are held in high regards by colleagues and FDA.
- B. We have an international network of collaborators in place.
- C. GP has not taken any steps in this regards and is poorly regarded in view of their aggressive, poorly prepared, rushed first steps.

This email extolling EGT's accomplishments, dispels any notion that the performance by EGT under the 2005 Agreement was anything other than exemplary.

53. In June 2010, EGT President Sam Salman and Patrick Girondi visited Neil Exter, a principal of Third Rock, Mitch Finer, Chief Scientific Officer of Bluebird, and Nick Leschly, CEO of Bluebird. Leschly was formerly Business Development Officer of Agios Pharma.

54. Because of the disastrous Bluebird French trials in 2009, EGT wanted a guarantee of the use of the EGT Vector as a condition to negotiations. Leschly refused and negotiations halted.

55. On June 22, 2010, Andrew Maslow, SKI Director of the Office of Technology Development, sent an email to EGT confirming his belief that EGT's technology was superior to the Bluebird technology and commending EGT for years of effort to develop the EGT Vector.

56. Maslow subsequently informed EGT that as the result of exemplary work of EGT in developing Thalagen, the SKI Board was interested in creating a center of excellence at SKI offering boutique gene therapy with a targeted cost of up to \$650,000 per patient. Maslow discussed the ability of EGT to send wealthy patients from such countries as India, Italy and Greece who could afford the treatment. This strategy represented many millions of dollars in new revenue that could be generated for SKI.

57. EGT had transformed the Vector from a technology with little commercial interest into an extremely valuable property.

58. The final step to be completed prior to commencement of Phase I Clinical trials was an application with the FDA for an Investigative New Drug ("IND"). In order to file, EGT needed a clinical trial agreement and the laboratory notes for experimentation and toxicology studies which were performed by SKI for EGT.

59. EGT orchestrated the arrival of European doctors to learn the technology for an eventual trial in Europe.

60. Through the efforts of EGT, Thalagen was developed into a drug worth at least tens of millions of dollars and possibly hundreds of millions of dollars upon commencement of a credible clinical program determining its safety in humans.

61. On August 1, 2010, it was reported on the front page of the *Wall Street Journal* that Dr. Craig B. Thompson was named CEO of SKI. Such appointment provided Bluebird with the opportunity to obtain the EGT technology for free and eliminate EGT, which was years ahead of Bluebird, as a competitor

62. Dr. Thompson was the scientific director of the Abramson Family Cancer Research Institute (LMAFCRI) at the Abramson Cancer Center of the University of Pennsylvania, from 1999 to 2010.

63. Thompson has substantial long-standing ties and business relations with the executives and investors of Bluebird.

64. In 2007, Dr. Thompson was a founder of Agios Pharmaceuticals (“Agios”).

65. In 2008, Third Rock Ventures invested \$33,000,000 in Agios.

66. Nick Leschly was a partner and founding member of Third Rock Ventures in 2007. Mr. Leschly played an integral role in the overall formation, development and business strategy of several of Third Rock’s portfolio companies, including Agios Pharmaceuticals, Inc. He worked as Agios Business Development Officer from 2009-10. In September 2010, Mr. Leschly became Bluebird Bio’s Chief Executive Officer and has been in this role ever since.

67. Celgene, a major biotech company, is the major investor in Agios and a major investor in Bluebird.

68. At the time Thompson was announced as the new CEO, EGT was poised for clinical trials of the EGT Vector on thalassemia patients at SKI and the National Institutes of Health (“NIH”). With Dr. Thompson in charge, SKI ignored the needs of millions of Thalassemia and

Sickle Cell Disease patients to engage in a fraudulent scheme to stop EGT's clinical trials and transfer EGT's technology to Bluebird.

69. SKI ignored the warnings of its executives and scientists that were heeded by EGT in rejecting any deal with Bluebird would shelve the EGT/SKI gene therapy and that Bluebird would proactively sabotage all the efforts of SKI and EGT.

70. SKI ignored the warning from its chief scientist after at least one meeting with Bluebird, that Bluebird was committed to proceeding with its inferior vector, stating that Genetix/Bluebird "lied" and "bluffed" about its vector's results and had bungled its first patient, and stating:

It is baffling that they would even continue with its vector, as admitted by Reilly-Finer at the meeting. The fact that leukemia may develop from the repopulating clone is somewhat lost in the shuffle. Bluebird will try to distance itself from that mess (new name, etc) but their vector as is still a lemon. There is a big machine behind this.

71. Discovery has revealed that under new management, SKI entered into a secret partnership with Bluebird for the purpose of fraudulently inducing EGT to transfer to SKI the physical EGT Vector and all of EGT's know-how, proprietary information and exclusive rights to patented technology (the "EGT Intellectual Property"). Pursuant to a secret agreement with Bluebird, SKI agreed to give the EGT Intellectual Property to Bluebird for free and share all results of clinical trials to allow Bluebird to develop a safe vector (hereinafter the "Secret Agreement"). The Secret Agreement provided that SKI would be totally reliant on Bluebird for funding of clinical trials and manufacture of vector, while providing that Bluebird had absolutely no obligation to provide the funding. Bluebird would have the ability to use all of EGT's Intellectual Property to create a safe Bluebird vector and then shelve the EGT vector by refusing to provide any funding. This was contrary to all the prior discussions concerning the necessity to maintain a competitive advantage over Bluebird to obtain Orphan Drug Exclusivity.

72. EGT did not know and could not have discovered such secret dealings until the production of documents in this lawsuit. EGT could not imagine that the esteemed and storied non-profit hospital and research center, with whom it had partnered for many years to develop the nascent science of gene therapy, would ignore the needs of millions of Thalassemia and Sickle Cell Disease patients to engage in a fraudulent scheme to stop EGT's clinical trials and to take possession of EGT's Intellectual Property solely to benefit Bluebird and its investors. EGT could not imagine that SKI would enter into such an insidious agreement and abandon its scientists, and the brilliant cure that they had developed.

#### **Securing Possession of EGT Vector**

73. In late September of 2010, SKI was faced with a quandary, as everything was on track to commence trials with the EGT Vector. On September 27, 2010, an internal SKI email confirmed that "The protocol under which the EGT vector will be used has passed res. council. .the protocol is in the queue for IRB approval."

74. In order to carry out the secret partnership between SKI and Bluebird it was necessary to halt the imminent EGT clinical trials. Bluebird's clinical trials had been halted by the French and US governments and Bluebird was three years away from resuming clinical trials. Successful clinical trials by EGT would make it impossible to transfer the EGT Information to Bluebird for free and make it impossible to eliminate EGT as Bluebird's competition for a Thalassemia cure.

75. The first step to halting clinical trials was to secure possession of the physical EGT Vector.

76. An SKI email discusses the importance of the physical EGT Vector and devising a strategy to gain control of the physical vector, which EGT owned and had paid for:

First of all, EGT owns the vector stock we (desperately) need to open the trial, hopefully within a few months. If we lose this batch, it will be at least 18-24 months before another batch can be made and released, not to mention that we would need at least 500K (in-house cost), or much more elsewhere, to have it manufactured. . . It therefore seems appropriate to put pressure on EGT, but not to simply terminate the relationship, which alone, won't get us anywhere – and will even cost us the impending trial if they do not give us the vector. Securing access to the already made batch of vector is of paramount importance. . .

77. A joint plan was implemented by SKI and Bluebird to wrest the physical EGT Vector away from EGT.

78. In September of 2010, SKI urgently requested that EGT deliver the Vector to SKI to complete a mobilization study. EGT delivered to SKI the Vector, in an amount sufficient to treat 7-10 patients. The Vector was in place for the commencement of clinical trials in December 2010. The Vector was irreplaceable. No similar medicine existed anywhere in the world. EGT instructed SKI that it could use a small portion of the Vector to test in live patient cells and to complete the mobilization study.

79. The Vector was delivered to SKI based on the understanding that a part of the Vector was to be used to perform clinical trials on patients at SKI. A portion of the Vector was to be delivered to the project principal investigator Dr. John Tisdale (“Tisdale”), Senior Investigator at the NIH Molecular and Clinical Hematology Branch, to treat NIH patients. Dr. Tisdale and his Branch are recognized as a center of excellence for developmental clinical activities using viral vectors for the delivery of gene therapies to treat hemoglobinopathies and specifically Sickle Cell Diseases.

80. The transfer of the vector was a ruse to secure physical possession of the EGT Vector to SKI under false pretenses by claiming that it was urgently needed at SKI to complete the mobilization study necessary for EGT to conduct clinical trials. The actual purpose was to secure possession of the Vector to prevent EGT from conducting clinical trials at NIH or elsewhere



and to allow SKI to conduct clinical trials only at SKI so it could share all the information from the clinical trials with Bluebird.

**SKI Makes Outrageous Demand For Money That Was Not Needed**

81. Discovery has revealed that this demand was a ruse to halt clinical trials as no such payment was required under the 2005 Agreement and SKI knew that no such payment was needed.

82. A September 27, 2010 email confirmed that SKI had received a third-party grant to pay for clinical trials on the first three patients, stating: “Michel [Sadelain] has received sufficient funding . . . to treat three patients. EGT is not yet made aware of this.”

83. In another September 27, 2010 email Maslow stated that EGT had sufficient funds to proceed with clinical trials: “EGT now has \$400,000 for the first patient and, after we sign a clinical trial agreement with them (the final draft was given to us today), they will be able to get a letter of credit for \$1.2 million.”

84. Taking marching orders from the newly-arrived CEO, Craig Thompson, on October 19, 2010, Maslow attempted to halt the EGT clinical trials by demanding from EGT four million in cash in advance (\$400,000 per patient for up to 10 patients) before it would allow any clinical trials at SKI. Maslow offered to treat Patrick Girondi’s son, Rocco Girondi for free if EGT acceded to all of SKI’s demands.

85. EGT was unwilling to pay to SKI more than eight times the cost at which patients would be treated at NIH and unwilling to pay in advance before it even knew how many patients would be tested. EGT informed SKI that the first patient would be treated at SKI for \$400,000, and that the remaining patients would be treated at NIH for less than \$50,000 per patient with Principal Investigator Dr. John Tisdale.

**SKI Transfers EGT Intellectual Property to Bluebird in 2010**

86. On November 2, 2010, a secret meeting between SKI and Bluebird was held, at which SKI made a technical presentation regarding the EGT Vector, disclosing to EGT's sole competitor, confidential and proprietary information and trade secrets of EGT and SKI to enable Bluebird to improve its vector. SKI had no right to disclose the EGT Intellectual Property and had no right to disclose SKI intellectual property that was subject to the exclusive license to EGT under the 2005 Agreement, which unquestionably remained in full force and effect in November 2010. Disclosure of such information to EGT's sole competitor, to whom EGT had refused to sell the same information, is nothing less than an abomination.

87. Additional secret meetings were held by SKI and Bluebird to transfer EGT Intellectual Property to Bluebird (at least on February 16, 2011 and June 4, 2011). Discovery has further confirmed that the disclosure included "trade secrets, know-how, inventions, technical data or specifications, testing methods, business and financial information and research and development" as stated in an agreement to memorialize the disclosure which was signed by Bluebird on November 21, 2011.

**SKI Demands Then Rejects a Larger Development Partner**

88. Unsuccessful in its \$4,000,000 extortion demand, and with the disclosure of the EGT Intellectual Property well under way, SKI halted the clinical trial process by refusing to deliver the EGT Vector to NIH to commence clinical trials. SKI stated that it would not return the EGT Vector and/or allow clinical trials to proceed unless EGT secured a larger pharmaceutical company as a partner for future commercial development, even though the Vector had not yet been tested on a single patient. Discovery has revealed that this demand was also a ruse in as much as SKI had already entered into the Secret Agreement with Bluebird.

89. EGT turned to Sigma Tau as a tentative partner for commercial development. Sigma Tau is a pharmaceutical company that had for years supported the EGT mission. It is an orphan disease company with over \$1 billion in annual sales and fifteen approved drugs for orphan indications.

90. On November 22, 2010, EGT met with Maslow and officers of Sigma Tau to confirm that Sigma Tau was a suitable partner for commercial development. Maslow started the meeting by stating that he had just come from a meeting with Dr. Thompson to make clear to all participants that he was acting under the direction of Dr. Thompson to whom he directly answered. Maslow stated to the Sigma Tau officers that: “The technology does not work. If I were you, I would not invest in it.” Maslow knew this statement to be false and made it as part of the Secret Agreement with Bluebird.

91. On November 23, 2010, SKI claimed that EGT was in breach of the 2005 Agreement for an alleged past due license fee of \$400,000 for Sickle Cell Disease. The payment had been waived by SKI in 2008 and Maslow’s complimentary letter of June 22, 2010, made no mention of any arrears. Nonetheless, EGT made the \$400,000 payment on December 7, 2010.

92. Dr. Thompson’s involvement in the Secret Agreement with Bluebird is confirmed by his request for a meeting with SKI scientists held on December 2, 2010 to discuss Bluebird and EGT. At such meeting, Dr. Thompson was warned by SKI scientists: “Don’t be duped by apparent interest” of Bluebird.

93. Dr. Thompson’s involvement in the Secret Agreement with Bluebird is further evidenced by his attendance on December 7, 2010, at an important meeting of SKI’s Committee on Technology Transfer, though he was not on the Committee, at which the termination of the EGT and the agreement with Bluebird was discussed. EGT attempted to remove the trail of fraud by deleting the reference to discussions of EGT and Bluebird from the official minutes.

94. In January 2011, EGT signed a contract with Sigma Tau to act as the commercial partner demanded by SKI which would have been lucrative for SKI and EGT. The contract provided for a \$3 million down payment, the right to purchase 20% of EGT's shares based on a valuation of the EGT Intellectual Property of \$20 million, full funding of all development and Clinical Trial costs, and 22-24% royalties on the final product.

95. The Sigma Tau contract was submitted to SKI for approval, as required by the 2005 Agreement. It was immediately rejected by SKI.

#### **SKI Fraudulently Induces EGT To Sign The 2011 Agreement**

96. EGT sought every possible solution to put the trial back on track. After EGT refused SKI's offer to purchase the Vector project for a payment of \$3 million, SKI commenced an arbitration on January 21, 2011, claiming that EGT had been lacking in its development duties. This is less than six months after SKI praised EGT for its fine work.

97. On March 23, 2011, EGT arranged for a special courier pick up of the EGT Vector from SKI to have it delivered to Dr. Tisdale at NIH. SKI refused to return the EGT Vector.

98. On June 16, 2011, EGT and SKI met at the office of SKI counsel to discuss the resumption of clinical trials. Approximately ten (10) months had passed since the EGT Vector was delivered to SKI. Over seven months had passed since the new SKI senior management started the process of trying to take the EGT Vector and preventing the filing of an IND Application with the FDA and the treatment of patients.

99. The June 16, 2011 meeting was attended by Pat Gironi and Sam Salman on behalf of EGT and Maslow on behalf of SKI. SKI proposed a new agreement which would give SKI control of the clinical trials and commercial exploitation of the EGT Vector.

100. At the June 16, 2011, meeting Maslow knowingly made false statements to Girondi and Salman to induce EGT to enter into a new agreement with SKI. Maslow stated that SKI “had spent \$1,500,000 to write the IND.” Maslow stated that the “the IND is done and ready to be filed immediately.” Maslow repeated this statement several times. Maslow further stated “the first patient will be treated no later than October 2011.”

101. SKI failed to disclose the Secret Agreement with Bluebird.

102. SKI failed to disclose the secret meetings with Bluebird to transfer the EGT Intellectual Property to Bluebird to allow Bluebird to improve its vector.

103. SKI failed to disclose the warnings to SKI from its executives and scientists that Bluebird intended to put the EGT Vector on the shelf and sabotage the EGT Vector.

104. SKI failed to disclose that it did not intend to make any commercially reasonable efforts to exploit the EGT Vector and it had already agreed to enter into an agreement with Bluebird that would assure that the EGT Vector would be killed.

105. SKI failed to disclose its Secret Agreement with Bluebird that was for the sole benefit of Bluebird and its investors under which Bluebird would have no obligation to fund any clinical trials or vector manufacture or other development if the EGT Vector, yet would be able to incorporate all EGT Intellectual Property into the Bluebird Vector.

106. SKI failed to disclose its Secret Agreement with Bluebird under which Bluebird would be the sole source for funding of trials and development of the EGT Vector, yet Bluebird would have no obligation to provide any funding.

107. Such facts could not have been discovered by any reasonable diligence.

108. Such facts are so outrageous that neither EGT nor any reasonable person could imagine that SKI has entered into such a heinous agreement with EGT’s sole competitor.

109. Based on the representations described herein, and SKI's failure to disclose material facts known only to SKI, EGT entered into the agreement with SKI dated June 17, 2011 ("2011 Agreement"), a copy of which is attached hereto as Exhibit B.

110. The 2011 Agreement provided for a sale, assignment and transfer to SKI of the EGT Intellectual Property for a short-term trial on three patients, evaluation of the results and gave SKI discretion to determine whether or not it would proceed further with the project which was stated to have a limited, but not specifically identified, term.

111. In return, under the 2011 Agreement, SKI would: (i) file the IND with the FDA the following day, (ii) use commercial standards to bring the project forward and fund the Clinical Trials and (iii) "use its best efforts to seek a medically reasonable determination as to the efficiency of the vector, based on the data collected for the first three patients."

112. The 2011 Agreement also provided that SKI shall pay EGT fifty percent of any consideration derived from any exploitation of the Vector. SKI never disclosed that it agreed to pay EGT the fifty percent because the Secret Agreement with Bluebird assured that the EGT would receive fifty percent of zero.

113. The 2011 Agreement, imposed by SKI, was of limited duration. The 2011 Agreement provided, at paragraph 9, that SKI was to "keep full, true and accurate books of account containing all particulars that may be necessary for the purpose of showing the amounts payable to EGT hereunder."

114. The 2011, Agreement further states: "For the term of this agreement, upon reasonable written notice, SKI shall allow EGT or its agents to inspect such books and records for the purpose of verifying SKI's payments." (Emphasis added). In 2011, EGT was years ahead of Bluebird.

#### **The Deadly Agreement**

115. On November 21, 2011, SKI signed an Option Agreement and a Research Collaboration Agreement with Bluebird to memorialize what had been secretly agreed to in 2010. The conspiracy to defraud EGT into transferring the EGT Intellectual Property resulted in a “deal” that was the ultimate sweetheart deal to benefit only Bluebird and its investors and kill the development of the EGT Vector. Bluebird was not even required to purchase the EGT Vector to shelve it (which EGT had refused to allow). SKI scientists later accurately referred to the agreement with Bluebird as the “Deadly Agreement” which Plaintiff will adopt for purposes of this Complaint

116. The Deadly Agreement:

- gave to Bluebird all EGT Intellectual Property and all SKI technology and know-how and all results from clinical trials of the EGT Vector, for free;
- provided Bluebird with the consulting services of SKI scientists to improve the Bluebird vector and incorporate the EGT Intellectual Property into the Bluebird vector, for free;
- did not require Bluebird to fund any clinical trials or vector manufacture of the EGT Vector or provide any significant funding to develop the EGT Vector;
- gave Bluebird an exclusive long-term option to license the EGT Vector at a deeply discounted price; and
- which made it impossible for SKI to obtain funding from any other source to fund clinical trials of the EGT Vector.

117. In June 2012, an SKI internal review of the Deadly Agreement provided a succinct summary of the legion of one-sided provisions intended to give Bluebird the power to kill development of the EGT Vector. The following summaries are direct quotes from the SKI memo including all “Implication” paragraphs:

\* \* \*

- BBB granted right of first refusal to any MSKCC inventions relating to the development of any lenti-viral based gene therapy for hemoglobinopathies.

\* \* \*

- BBB is given access to data and reports arising from collaboration but this obligation is not reciprocal (e.g. BBB is not contractually bound to provide any data to MSKCC).

- **Implication:** MKSCC may not be fully aware of BBB's work in this area and how far they are advancing their own related projects in comparison to the MSKCC vectors/projects

\* \* \*

- BBB granted NERF [non-exclusive royalty-free license] to use results and reports obtained in connection with collaboration for any purpose (right is not reciprocal)

\* \* \*

- BBB is granted an exclusive option [3 year and 3 month] to any inventions [including results and data] relating to hemoglobinopathies developed by MSKCC, or jointly with BBB, whether under the collaboration or outside of it.

- **Implication:** . . . Pre-set license terms are extremely low, RR [royalty rate] 2-4.5% (with 50% stacking) and 0-5% sublicensing revenue... (they can potentially avoid need to pay any sublicensing revenue). Also, milestones are quite low (and back-loaded).

- Development of "Best Vector." BBB reserves the right to develop (at their sole discretion) their proprietary gene therapy vector in parallel with the Exclusive Collaboration. If BBB "elects" not to "develop" **Collaboration IP** . . BBB required to return Collaboration IP.

- **Implication:** This language is a bit grey in that there are no hard and fast milestones to validate that BBB is actually "developing" the Collaboration IP and it is at BBB's election to return MSKCC's right on the Collaboration IP. Therefore, BBB could drag its feet in development of the Collaboration IP to effectively sideline MKSCC while it advances its own competing product virtually assuring MKSCC would be unable to secure another development partner.

- BBB is granted a NERF [nonexclusive royalty-free license] to "Know-How" which encompasses information, material and data developed during



the collaboration or outside of it that relates to lentiviral gene therapy for hemoglobinopathy.

- **Implication:** Technically BBB now has the right to sell MKSCC data (which may include clinical data as the collaboration may encompass clinical studies)
  - BBB can terminate the agreement without cause but this right is not reciprocal.
  - Exclusive Option to SK 972, TNS9.55.3 (and related modifications necessary for using vector to treat sickle cell disorder), modifications to TNS9.55.3 (to the extent a new IND would not be required), any other vector that was part of the EGT License Agreement, and any other vector solely created by MSKCC prior to the Effective Date of the Option Agreement.
- \* \* \*
- Option expires on the 2nd anniversary of dosing of the 3rd patient in MSKCC's current clinical trial relating to TNS9.55.3

118. The Deadly Agreement was far less favorable than the Sigma Tau agreement rejected by SKI in January 2011. The Sigma Tau contract provided for a \$3 million down payment, Bluebird paid zero. Sigma Tau provided full funding of all development and Clinical Trial costs. Bluebird paid zero. Sigma Tau provided for 22-24% royalties on the final product. Bluebird provided for 2-4.5% royalties with a possibility of no royalties on sublicensing.

119. The secret partnership entered into in 2010 culminated in the Deadly Agreement which assured that the EGT Vector would never be commercially exploited in competition with Bluebird. After transferring the EGT Information to Bluebird to allow Bluebird to develop a safe vector and re-start its clinical trials, the EGT Vector was abandoned by Bluebird. SKI was able to perform highly successful clinical trials on three patients, and share the results with Bluebird, and was thereafter SKI was precluded by the Deadly Agreement from obtaining any commercial funding to continue the clinical trials.

120. In an August 20, 2012 SKI email, an incredulous SKI executive asked: “Who negotiated this deal with Bluebird bio on our end? Did we use any outside counsel?” An SKI employee responded “Viviane Martin with I am assuming input from Andy Maslow. To my knowledge, no outside counsel was involved.”

121. A July 9, 2012 email confirms that SKI lead scientists had been consulting with Bluebird and that “We work for bbb for free.”

122. SKI scientists were furious once they realized that their Nobel-prize caliber achievement embodied in the EGT Vector had been sabotaged by the deal with Bluebird. An August 27, 2012 email stated:

I think that the meeting made it plain that bbb has obtained a lot of information and future rights in exchange for next to nothing. . . Although they have already pillaged most of our know-how and strategic ideas, I would like to know whether this agreement can be rescinded.

123. A November 28, 2012 email from an SKI scientist states:

Simply put, they benefited from our expertise as they were getting started (getting our full IND before submitting their own and all of our ideas for a song) and later sat on our samples delaying our IND by several months. They are pouring \$ into other centers, while we struggle moving forward. They are vigorously pushing their vector ahead after having in effect neutralized our ability to seek new partners.

124. On March 12, 2013, Eric Raskin explained to Eric Cottingham, SKI’s head of Research and Technology Management, explained the result of the conspiracy that resulted in the Deadly Agreement, leaving the EGT Vector on a shelf:

While Bluebird recently raised funding, and can produce its vector and fund its own clinical trials, the agreement in place between BBB and MSKSCC [SKI] prevents MSKCC from finding other sorts of funding for vector production and clinical trial completion.

125. On July 5, 2013, Dr. Sadelain summed up the impact of what he called the “deadly agreement”:

\* \* \*

By the way, bbb is also widely perceived as a bully with no interest in the patients whatsoever.

\* \* \*

We also know that our vector expresses better than that bbb's, but I assume they are working on a better one that will circumvent our patent.

\* \* \*

Because they will utilize myeloablative conditioning in patients with not-too-severe thalassemia, bbb will likely obtain a good clinical result in their first 1-2 patents (i.e. abolished transfusion need at 6 months). They will have these data in the spring of 2014. By that time, we will not yet be 1-year post Pt 3 (July 2014). Their slowing down of our IND submission will have paid off for them.

I predict they [Bluebird] will rush to shop their globin program to big pharma next spring, having done everything possible to ensure that we cannot do the same.

In summary, we have a better vector, very promising data, and tons of sympathy and support other-than-financial in the thalassemia community at the international level. Without the deadly agreement that holds us back, we would be actively seeking a commercial partner right now.

126. A July of 2014 internal SKI email expressed disgust at the lack of resources devoted to the project:

We would have had these results over a year ago if we had 1M [\$1 million] for vector studies (refining production and pay for the manufacture of a high quality cGMP lot). The issue is simple: we have a budget of 400K per year and they raised 140M dollars. We could not affors[d] top of the line vector manufacturing. This is both disgusting and depressing.

#### **SKI and Bluebird Kill the Project After Successful Clinical Trials**

127. SKI's IND was not filed until September 2011, and then was rejected by the FDA as not ready for submission.

128. An internal email of SKI confirms that the SKI IND was delayed for several months because Bluebird delayed the return of SKI samples (which had been paid for by EGT) needed to file the IND.

129. The IND was finally accepted by the FDA in or about June 2012. The first patient was treated in November of 2012. The second and third patients were treated in February 2013 and July 2013.

130. An internal SKI email states “We infused today, July 29, 2013, the third patient on MSKCC’s thalassemia trial with TSN9.3.55 . . . This is an important milestone toward our liberation from the prison bbb locked us in.” Such date is a reference to the fact that bbb exclusive rights to license the EGT Vector and block EGT from obtaining any other commercial funding started to run on July 29, 2013.

131. SKI presented positive data on the clinical trial at the American Society of Hematology meeting in December of 2013. SKI further published the optimal results of its stem cell mobilization procedure in ‘Blood’ magazine in March of 2014.

132. The treatment of three patients in clinical trials achieved precisely the goals that they were intended to achieve. As stated in the IND filed by SKI with the FDA and published on [clinicaltrials.gov](http://clinicaltrials.gov), the “Primary Outcome Measures” of the clinical trials on the first ten patients were Safety and Tolerability of the gene therapy. The Primary Outcome Measures were unquestionably achieved as the gene therapy was proven safe and tolerated by the patients.

133. The Secondary Outcome Measures – Level of Engraftment and Frequency of Post-Transplant Palliative Transfusions were also unquestionably achieved. Patients showed gene expression and significantly reduced their need for transfusions which ameliorates their medical condition and extends their life expectancy.

134. No document produced by SKI during discovery questions the achievement of the Primary Outcome Measures and Secondary Outcome Measures of the Phase I clinical trials on three patients.

135. According to the 2011 Agreement, in pursuance of diligent and commercial standards, SKI was obligated to proceed. It has been over three years since SKI treated a patient. EGT's competitor, Bluebird, has treated at several patients in such time period.

136. The 2011 Agreement incorporated the requirements of Article 3.1 of the prior 2005 License Agreement which provides as follows:

“3.1 LICENSEE and its Sublicensees shall use commercially reasonable efforts to develop and seek registration for, and to introduce into the market, Licensed Products in Field A and in Field B through an appropriately thorough and diligent program for exploitation of the Patent Rights as detailed in the Plan referred to below, consistent with sound and reasonable scientific and business judgment, and thereafter continue active, diligent marketing efforts for one or more Licensed Products through the life of this Agreement.”

(copy of 2005 Agreement attached hereto as Exhibit A).

137. The fact that SKI achieved the desired results of the clinical trials, presented positive data resulting in no adverse effects and reduced the need for transfusions obligated SKI to “use commercially reasonable efforts to develop and seek registration for, and to introduce into the market,” the EGT Vector.

138. SKI has not treated any patient for this clinical trial in over three years. SKI, by its own admission, has not been able to replicate the EGT Vector. SKI, by its own admission, lacks funding for the project.

139. SKI never disclosed to EGT the existence of the Deadly Agreement which made Bluebird the sole source of funding for its clinical trials. SKI never disclosed to EGT that it had abandoned the project, and that EGT was prevented from securing funding from any other commercial source.

140. As intended by the secret partnership between Bluebird and SKI in 2010, Bluebird was given the right to decide whether to fund the EGT Vector or allow it die.

141. Bluebird decided to develop its own vector using the EGT Intellectual Property and all of SKI's know-how and the exclusive consulting services of SKI's chief scientists, all of which they were given free of charge. SKI and Bluebird put the EGT Vector "on a shelf" and left it to die after the third patient was treated in 2013, allowing Bluebird to continue its clinical trials with no competition.

142. SKI, by its own admission, has rejected lucrative offers of funding from others to support or acquire this project.

143. In the spring of 2013, Eric Cottingham ("Cottingham"), the head of Research at SKI, claimed that the fourth patient would be treated within two months. He also informed EGT that the IND for the Sickle Cell Disease Trial would be written and that the Standard Operating Procedures would be delivered to Dr. Tisdale for the commencement of the Sickle Cell Trials at NIH.

144. EGT informed Cottingham that Bluebird was working on another version of their first vector and that SKI must move diligently to ensure that the best medicine eventually got to the patients. EGT made such statement with no knowledge that SKI had been working with Bluebird to develop its vector since 2010.

145. In June 2013, SKI removed Dr. Tisdale as Principal Investigator. Since the writing of the protocol in 2005 it was understood that Dr. Tisdale and the NIH were solely equipped to advance the gene therapy clinical trials for Sickle Cell Disease.

146. EGT continued to support the project and authorized transfer of the cell bank material (ingredients used in the production of the EGT Vector) to SKI.

147. In October of 2013, SKI informed EGT that all of the EGT Vector had been consumed in only three of the intended seven patients, and that SKI had analyzed and verified promising results. SKI stated that it had decided to produce more EGT Vector.

148. In November of 2013, EGT received from the Indian Thalassemia Foundation various emails sent by SKI attempting to charge patients for the clinical trials. EGT advised SKI that charging for clinical trials without explicit written permission of the FDA damaged the project. Further EGT notified SKI that it was their responsibility to fund said clinical trials.

149. By November 2014, SKI had told EGT and the project supporter, Cooley's International, that SKI had no EGT Vector to continue and would no longer fund the project. It had been over three years since SKI had fraudulently induced EGT to enter into the 2011 Agreement, and now SKI claimed that the project was dead.

150. As a result of SKI delays in conducting the clinical trials, the project risks losing the critical Orphan Drug Designation to Bluebird. The Orphan Drug Designation granted by the FDA guarantees market exclusivity to the designee.

151. The risk is that an inferior product will make it to patients and the market in the place of the superior EGT Vector.

152. In late 2014, Biomarin, a pharmaceutical company which was a fitting candidate, approached SKI and attempted to purchase the EGT Vector but was rejected by SKI.

153. In December of 2014, Cottington told Patrick Girondi that he had no idea if the fourth patient had been treated but that he was confident that a deal with some major pharmaceutical company would be completed by the end of the year that would move the project forward. Also in December of 2014, Juno Pharma did an IPO. Sadelain is a scientific founder of Juno. SKI received 2,000,000 shares of stock. This further confirms SKI's priority in cancer research.

154. On January 15, 2015, Cottington told Patrick Girondi that the fourth patient had not been treated and that he had no idea when or if the fourth patient would ever be treated. Cottington also told Patrick Girondi that the project was without funding and had no Vector remaining to treat

patients. He acknowledged that SKI may be forced to give the project back to EGT, but that would be his last choice.

155. SKI sent a letter to EGT dated February 18, 2015, that acknowledged that SKI is not actively pursuing exploitation of the EGT Vector. A copy of that letter is attached hereto as Exhibit C. At the same time, SKI has precluded EGT from any possible exploitation of the EGT Vector and summarily rejected overtures from companies expressing strong interest in providing major funding towards the commercialization of the EGT Vector.

156. After reporting positive clinical results, SKI has abandoned the EGT Vector. This represents a breach of contract by precluding development under the 2011 Agreement, further emphasized by the inclusion of Article 3.1 of the 2005 contract. Three plus years is unconscionably long in the shortened lives of the thalassemia patients waiting for EGT's Vector. It means dozens of blood transfusions (taking up to 8 hours each) and the resulting increase in harmful iron in each in the patient's bodies.

157. SKI's conduct caused EGT's counsel to send an email on February 23, 2015, noting that the License Agreement was for a limited term and that SKI was not actively pursuing exploitation of the Vector. That email concluded by stating that: "EGT hereby seeks immediate reversion of all right, title and interest in the Vector and EGT Information." A copy of the letter is attached hereto as Exhibit D.

158. In a letter dated March 6, 2015 (copy attached hereto as Exhibit E), SKI's outside legal counsel succinctly conceded that SKI is stonewalling any information concerning the advancement of the subject medical remedy:

"In any event, SKI has no obligation to keep your client informed of its development plans or studies."



159. EGT has invested in excess of \$9,600,000 in the development of the EGT Vector. EGT's competitor spent 50 times this amount to accomplish the same goals for an inferior product.

160. The EGT Vector represents ground breaking technology at the very forefront of field of gene therapy. It represents many years of work by brilliant scientists. It has great value as a cure for thalassemia and sickle diseases and great value to any pharmaceutical company wishing to enter into or jump to the front of the gene therapy field to treat other diseases.

161. The EGT Vector and other developing gene therapy treatments for thalassemia and sickle cell disease have repeatedly been the subject to valuation in the marketplace.

162. In January 2011, EGT entered into a contract with Sigma Tau that was rejected by SKI. It included an option to purchase EGT shares at a valuation of \$20 million, a \$3 million down payment, full funding of all development and Clinical Trial costs, and 22-24% royalties on the final product.

163. Sigma Tau entered into the contract despite Maslow's attempts to prevent a contract by telling Sigma Tau that "the technology does not work. If I were you, I would not invest in it."

164. EGT accepted the valuation for the shares of EGT because it was pressured by SKI to enter into such an agreement as a condition to commencing clinical trials.

165. The valuation in the Sigma Tau contract represented a deep discount of the value of EGT's rights in the EGT Vector in January 2011, prior to commencing any clinical trials of the EGT Vector. The value greatly increased after clinical trials were conducted on the first patients at SKI and the positive results were published by SKI.

166. The contract with Sigma Tau had been executed and required only the approval of SKI to be effective. SKI inexplicably and without any rationale or justification, summarily rejected the contract with Sigma Tau and the outside funding that SKI had arbitrarily required EGT to solicit as a condition to commencing clinical trials.

167. In 2013, investment banks valued EGT at \$40-60 million.

168. In March 2015, Sarepta Therapeutics approached SKI about buying the EGT Vector. Sarepta valued a 50% interest in the project at \$100 million. SKI refused to discuss a transaction without any rationale or justification.

169. Bluebird has raised approximately \$1.3 billion in capital. Bluebird is built primarily around development of gene therapy for thalassemia and sickle cell diseases.

170. In 2013, Bluebird raised \$116 million in an initial public offering of its shares based on a valuation of the company at \$800 million. In 2015, Bluebird raised \$477 million based on a valuation of \$7.8 billion. In 2017, Bluebird raised \$259 million based on a valuation of \$4.5 billion.

171. Analysts attribute at least 75% of such value to Bluebird's vector for thalassemia.

#### **FIRST CAUSE OF ACTION Fraud Against SKI**

172. Plaintiff repeats and realleges each and every allegation contained in paragraphs 1 through 171 as if fully set forth herein.

173. SKI never disclosed to EGT that it had entered into a secret partnership with Bluebird to wrest control of the EGT Vector and EGT Intellectual Property from EGT to eliminate EGT as competition with Bluebird.

174. SKI never disclosed to EGT that it had entered into the Secret Agreement to benefit only Bluebird.

175. SKI never disclosed that it had already began the transfer of the EGT Intellectual Property and all SKI know-how to Bluebird for free to allow Bluebird to develop its own vector and re-start clinical trials.

176. SKI never disclosed that its scientists were working for Bluebird for free to develop the Bluebird vector.

177. SKI never disclosed to EGT that it agreed that Bluebird would be the sole source of financing for the EGT Vector and Bluebird had no obligation to provide any financing.

178. EGT never disclosed that pursuant to the Secret Agreement, SKI secured possession of the physical EGT Vector to prevent EGT from commencing clinical trials.

179. EGT never disclosed that pursuant to the Secret Agreement, on October 19, 2010, SKI attempted to halt the clinical trial process by demanding \$4 million in cash in advance (\$400,000 per patient) to which it was not entitled. Maslow offered to treat Patrick Girondi's son for free if EGT acceded to all of SKI's demands. SKI failed to disclose that it had a grant to pay for the clinical trials. SKI used this ruse despite the fact that it knew EGT had funding for clinical trials.

180. When advised that EGT was unwilling to pay to SKI more than eight times the cost at which patients would be treated at NIH, SKI halted the clinical trial process by refusing to return EGT's Vector to NIH.

181. SKI refused to return the EGT Vector and/or allow clinical trials to proceed unless EGT secured a larger pharmaceutical company as a partner for future commercial development. SKI failed to disclose that it had already entered into an agreement with Bluebird.

182. EGT secured Sigma Tau which was introduced to SKI to its new partner. On November 22, 2010, Maslow stated to the Sigma Tau officers that: "The technology does not work. If I were you, I would not invest in it."

183. When EGT presented SKI with a signed contract with Sigma Tau that would fully fund all trials, it was rejected by SKI.

184. On November 23, 2010, SKI claimed that EGT was in breach of the 2005 Agreement for an alleged past due license fee of \$400,000 that had been postponed by SKI.

185. When EGT made the \$400,000 payment, SKI resorted to making knowingly false statements to induce EGT to terminate the 2005 Agreement.

186. On June 16, 2011, EGT and SKI met to discuss the resumption of the clinical trial process. Approximately ten (10) months had passed since the EGT Vector was delivered to SKI. Over seven (7) months had passed since new SKI management had begun blocking progress. They did so by failing to return the EGT Vector, preventing the filing of an IND Application with the FDA, and filing a demand for arbitration, thereby preventing treatment of patients.

187. The June 16, 2011 meeting was held at the office of SKI counsel and was attended by Patrick Girondi and Sam Salman on behalf of EGT and Maslow on behalf of SKI.

188. SKI demanded a new agreement which would give SKI control of the clinical trials and commercial exploitation of the EGT Vector.

189. At the June 16, 2011, meeting Maslow made the following knowingly false statements to Patrick Girondi and Salman to induce EGT to enter into a new agreement with SKI. Maslow stated that SKI “had spent \$1,500,000 to write the IND.” Maslow stated that the “the IND is done and ready to be filed immediately.” Maslow further stated “the first patient will be treated no later than October 2011.”

190. Each of those statements was known by Maslow and SKI to be false. Maslow fabricated the amount spent by SKI and knew that the IND application was not prepared to be filed. EGT had no ability to verify these statements as the information was solely within the control of SKI.

191. Maslow and SKI knew that it would be impossible to treat any patients by October 2011. Maslow knew and failed to disclose to EGT that the required approvals of SKI’s Internal Review Boards (“IRBs”) to allow the clinical trials to be performed at SKI had not yet been

received. SKI had previously demanded an advance \$4,000,000 payment from EGT for clinical trials that SKI was not yet approved to perform.

192. SKI knew that EGT would rely on such false statements and failure to disclose material facts to its detriment. SKI knew that as a result of the one year delay, which started with the entry of new SKI senior management that EGT would rely on such false statements because it would not endure further delays in the clinical trials.

193. SKI knew that there was a large group of Thalassemia patients and advocates that had supported EGT since its formation and that they were closely following the development of the EGT Vector and awaiting the commencement of clinical trials.

194. SKI knew that the delay caused by SKI's efforts to take EGT's Vector resulted in countless blood transfusions, painful chelation medication to remove the resulting iron, and years lost in already shortened life spans.

195. SKI knew that Rocco Girondi, the son of Patrick Girondi who had created EGT, was then twenty-one (21) years old. The average age of mortality of Thalassemic patients is twenty-eight (28).

196. SKI knew that at that time it would take EGT eighteen (18) months to make more EGT Vector to replace the Vector that SKI was refusing to release. Today, production can be achieved in approximately 4 months.

197. SKI knew that due to SKI's actions, it would take EGT months to file an IND application.

198. SKI knew that further delay of the clinical trials would allow EGT's chief competitor to narrow the gap with EGT. At that moment, EGT was two years ahead of the Bluebird competition who had, as previously stated, abandoned their first vector in 2012.

199. Based on the exceedingly material representations that SKI had spent \$1,500,000 on the IND, and that it would file the IND immediately, and treat patients by October, 2011, EGT acceded to the agreement with SKI dated June 17, 2011 (“2011 Agreement”), a copy of which is attached hereto as Exhibit B. EGT entered into the 2011 Agreement in reliance on the long history of SKI as one of the leading research centers and hospitals. EGT entered into the 2011 Agreement in reliance on the fact that SKI was a nonprofit organization devoted to bringing the best care to its patients. EGT entered into the 2011 Agreement in reliance the stated mission of SKI to explore new and promising ideas without delay, and always look for the fastest way to move research innovations toward patient benefit. EGT entered into the 2011 Agreement based on its knowledge that Michel Sadelain was a brilliant and highly-principled scientist who was totally dedicated to bring to EGT Vector to patients as quickly as possible. EGT entered into the 2011 Agreement based on the fact the representations were made by an attorney who was also SKI’s Director of Office of Technology Development.

200. SKI had a duty to disclose and failed to disclose the Secret Agreement between Bluebird and SKI to eliminate EGT as competition to Bluebird, including the facts set forth in paragraphs 178-190 above.

201. SKI’s superior knowledge of the essential facts of SKI’s Secret Agreement with Bluebird rendered the transaction embodied in the 2011 Agreement inherently unfair. These material facts were peculiarly within the knowledge of SKI. This information was not such that it could have been discovered by EGT through the exercise of any due diligence or ordinary intelligence. EGT did not have the means available to it to learn of the Secret Agreement.

202. EGT would not have entered into the 2011 Agreement if SKI had not made those false statements.

203. EGT would not have entered into the 2011 Agreement if SKI had disclosed the Secret Agreement with Bluebird. EGT rejected a deal with Bluebird in 2010 because it could not take the risk that Bluebird would purchase the superior EGT Vector to shelve it and eliminate competition.

204. EGT was damaged as the result of those false statements and failure to disclose material and special facts. Almost six years have passed. SKI had previously successfully infused three patients with very promising results and then abandoned the Vector.

205. SKI has done nothing during the past three years, during which time Bluebird has treated at least eight (8) thalassemia patients and passed EGT in the race for Orphan Drug Designation and the market exclusivity associated therewith.

206. SKI should not be permitted to preclude development of the EGT Vector, which would likely create a situation in which a less patient safe vector would receive exclusive Orphan Drug Designation and FDA approval.

207. EGT is entitled to damages in the amount to be proven at trial, plus punitive damages in an amount necessary to deter future misconduct by Defendant.

**SECOND CAUSE OF ACTION  
Breach of Contract Against SKI**

208. Plaintiff repeats and realleges each and every allegation contained in paragraphs 1 through 207 as if fully set forth herein.

209. SKI breached its obligation under paragraph 4 of the 2011 Agreement to “promptly file an investigational new drug application (IND) for Vector” with the FDA. Rather than filing immediately upon signing the agreement in June 2011 as represented to EGT, SKI filed an application in September 2011, which was rejected and returned by the FDA as incomplete.

210. SKI breached its obligation under paragraph 4 of the 2011 Agreement to diligently proceed with clinical trials on three patients. Rather than commencing trial not later than October 2011 (four months later) as represented to EGT, SKI treated the first patient in November 2012, (thirteen months later). SKI did not treat the second and third patients until February 2013 and June 2013. The two years that it took SKI to perform the clinical trials on three patients was not diligent. EGT's competitor Bluebird diligently proceeded with its clinical trials of gene therapy on Thalassemia patients and treated eight (8) patients within sixteen (16) months.

211. SKI breached its obligation under paragraphs 4 and 5 of the 2011 Agreement to treat patients "in accordance with the IND protocol." SKI attempted to require Indian patients to pay for the treatment. The 2011 Agreement mandated that SKI pay for clinical trials. The FDA requires explicit approval to charge patients in a clinical trial.

212. SKI breached its obligation under paragraph 4 of the 2011 Agreement that "SKI will provide to EGT trial information, as permitted by law". SKI failed to provide trial information to EGT despite there being no legal prohibition.

213. SKI breached its obligation under paragraph 5 of the 2011 Agreement to comply with the requirements of Article 3.1 of the 2005 License Agreement which provides as follows:

"3.1 LICENSEE and its Sublicensees shall use commercially reasonable efforts to develop and seek registration for, and to introduce into the market, Licensed Products in Field A and in Field B through an appropriately thorough and diligent program for exploitation of the Patent Rights as detailed in the Plan referred to below, consistent with sound and reasonable scientific and business judgment, and thereafter continue active, diligent marketing efforts for one or more Licensed Products through the life of this Agreement."

(copy of 2005 Agreement attached hereto as Exhibit A). SKI presented positive data resulting in no adverse effects and reduced patient's need for transfusions, yet has taken no further action,



which violates such obligations. SKI has failed to “use commercially reasonable efforts to develop and seek registration for, and to introduce into the market” the Vector.

214. SKI has not treated a patient in over three years. SKI, by its own admission, has not been able to replicate the EGT Vector. SKI, by its own admission, lacks funding for the project. SKI, by its own admission, has rejected lucrative offers of funding from others to support or acquire this project. There is no “appropriately thorough and diligent program for exploitation” of the very promising EGT Vector.

215. SKI breached its obligation under paragraph 5 of the 2011 Agreement that “SKI will use its best efforts to reach a medically reasonable determination of the efficacy of the vector, based on the data collected from the first three patients.” It is indisputable that the clinical trial on the first three patients achieved exactly the results that SKI and EGT desired: the EGT Vector was proven to be safe, resulted in gene expression, and in fact markedly reduced the need for blood transfusions in patients.

216. SKI has abandoned the clinical trials of the promising EGT Vector while Bluebird with its clinical trials sails towards exclusive orphan drug designation. SKI used all of the Vector produced by EGT and has failed to produce any more. SKI has informed EGT of its refusal to provide any more funding for the project yet, SKI has rejected offers from biotech companies to fund the clinical trials or to acquire the EGT Vector.

217. The foregoing actions are a breach of the duty of good faith and fair dealing implied in all New York contracts.

218. Abandonment of the EGT Vector and refusal to allow EGT to continue with the clinical trials would allow Bluebird to succeed with bringing its inferior vector to market before the EGT Vector. This would be a gross violation of SKI’s contractual duties to EGT and SKI’s

duties to the millions of Thalassemia and Sickle Cell Disease patients waiting for a cure of their deadly diseases.

219. EGT gave notice to SKI of its breaches of the 2011 Agreement.

220. SKI failed to cure its breaches.

221. SKI also breached the 2011 Agreement by entering into the Deadly Agreement with Bluebird intended to prevent commercialization of the EGT Vector.

222. Plaintiff EGT is entitled to damages in the amount to be proven at trial.

**THIRD CAUSE OF ACTION  
Breach of Contract Against SKI**

223. Plaintiff repeats and realleges each and every allegation contained in paragraphs 1 through 222 as if fully set forth herein.

224. On or about September 27, 2016, Plaintiff issued a subpoena to Bluebird in the action titled *Errant Gene Therapeutics, LLC v. Sloan-Kettering Inst. for Cancer Research*, No. 15-CV-2044 (AJN), a copy of which is attached hereto as part of Exhibit G. The subpoena sought the attendance by Bluebird CEO Nick Leschly at a deposition.

225. Bluebird responded by publicly filing a motion to quash the Leschly subpoena in the United States District Court for Massachusetts, a copy of which is attached hereto as part of Exhibit G.

226. Bluebird's public motion stated that Bluebird entered into the Secret Agreement with SKI. Such motion further stated that Bluebird was "EGT's main competitor."

227. The filing of the notice of motion was the first time that EGT learned that an agreement had been entered into by SKI and Bluebird.

228. The Secret Agreement was never disclosed to EGT by SKI.

229. The Secret Agreement was a breach of the 2005 Agreement and 2011 Agreement.

230. The disclosure of the EGT's Intellectual Property and SKI's intellectual property under exclusive license to EGT was a breach of the 2005 Agreement.

231. SKI took such actions to deprive EGT of the right to receive the fruits of such contracts in violation of the implied covenant of good faith and fair dealing.

232. EGT has been damaged by such breach.

233. Plaintiff EGT is entitled to damages in the amount to be proven at trial.

**FOURTH CAUSE OF ACTION  
Civil Conspiracy to Defraud Against SKI and Bluebird**

234. Plaintiff repeats and realleges each and every allegation contained in paragraphs 1 through 233 as if fully set forth herein.

235. SKI and Bluebird entered into a secret partnership and conspiracy for the purpose of wresting the rights to the EGT Vector from EGT and prevent completion of its clinical trials.

236. The partnership and conspiracy were undertaken to eliminate any competition to Bluebird in the development of a gene therapy for thalassemia and sickle cell diseases.

237. The partnership and conspiracy were undertaken to protect the \$1.3 billion investment in Bluebird based largely on the development of such gene therapy.

238. The conspiracy is rooted in the Orphan Drug Act (the "ODA") which provides a period of seven year exclusivity for the first company that develops a cure for an orphan disease. The conspiracy is rooted in the interlocking relationships between the executives of SKI, Bluebird, and Bluebird's investors.

239. The ODA provides for granting special status to a drug or biological product to treat a rare disease or condition upon request of a sponsor. Companies developing treatments for orphan drugs first receive Orphan Drug Designations. One company is eligible to receive Orphan Drug Exclusivity (ODE) which bars the FDA from approving any other application for the same drug

for the same orphan disease or condition for seven years. It bars the European EMEA from approving any other application for the same drug for the same orphan disease or condition for 10 years. By providing exclusivity, the program has opened the door to almost unlimited price tags for drugs for orphan diseases.

240. One of the world's first commercial gene therapies, Glybera, is approved in Europe for treatment of familial lipoprotein lipase deficiency (LPLD) at a cost of approximately \$1.6 million per patient.

241. JP Morgan reported that Bluebird estimates that Orphan Drug Exclusivity for a successful gene therapy for thalassemia patients will yield annual sales in excess \$1 billion. This does not include gene therapy for the larger pool of sickle disease patients (30 times larger in the United States).

242. In 2007, EGT obtained Orphan Drug Designation in the United States which provides the designee with a period of market exclusivity for an approved drug. In 2009, EGT was awarded Orphan Drug Designation in Europe.

243. At all relevant times, EGT has had one main competitor for development of a genetic therapy for hemoglobinopathies and for Orphan Drug Exclusivity – Bluebird (and its predecessor Genetix Pharmaceuticals).

244. Regardless of the award of Orphan Drug Exclusivity, the presence of a competitive gene therapy for the limited pool of Thalassemia patients in the US would greatly decrease the market value and potential revenue of the companies spending millions of dollars to develop a cure.

245. In March 2010, Third Rock Ventures acquired Genetix Pharmaceuticals for \$35 million and changed its name to Bluebird Bio Inc.

246. In March 2010, Third Rock approached SKI to purchase the EGT Vector. Such purchase would have eliminated Bluebird's competition. SKI referred Bluebird to EGT under the terms of the 2005 Agreement.

247. After meeting with Bluebird in June 2010, EGT informed Bluebird that its vector was inferior and that EGT would not make a deal unless Bluebird abandoned its vector and guaranteed that it would develop the EGT Vector. Bluebird refused.

248. Discussions of a purchase by Bluebird ceased because Mr. Girondi was not willing to take the risk that patients would be treated with an inferior therapy. Mr. Girondi was not willing to take the risk that Bluebird would purchase the EGT Vector just to eliminate competition for Orphan Drug Exclusivity and then abandon the EGT Vector.

249. On June 22, 2010, Maslow sent an email to EGT confirming his belief that EGT's technology was superior to Bluebird's technology and commending EGT for years of effort to develop the EGT Vector. *Exhibit L*.

250. Unable to acquire the EGT Vector, Bluebird entered into and engaged in a conspiracy and partnership with SKI to fraudulently wrest control of the EGT Vector from EGT to eliminate their competition.

251. On August 1, 2010 it was reported on the front page of the *Wall Street Journal* that Dr. Craig B. Thompson was named CEO of SKI.

252. Thompson has substantial long-standing ties and business relations with the executives and investors of Bluebird.

253. In September 2010, a conspiracy commenced to illegally halt clinical trials of the EGT Vector, to take possession of EGT's technology, eliminate Bluebird's competition and eliminate the threat to the investments of Thompson's partners in Agios. The conspiracy was carried out through the series of actions which includes those specified below.

254. Pursuant to the Secret Agreement between SKI and Bluebird, SKI urgently requested that EGT deliver the EGT Vector to SKI under the guise that it was needed to complete the mobilization study. The request was a ruse to gain possession of the EGT Vector and prevent clinical trials by EGT.

255. Pursuant to the Secret Agreement, on October 19, 2010, SKI blocked the clinical trials of the EGT Vector by demanding \$4 million in cash in advance (\$400,000 per patient for up to 10 patients). SKI made such demand knowing that it had received a grant to fund clinical trials and EGT had arranged funding for clinical trials.

256. In order to further delay clinical trials by EGT under the Secret Agreement, SKI demanded that EGT partner with a larger pharmaceutical company. SKI had already entered into the Secret Agreement with Bluebird.

257. Once EGT secured the interest of Sigma Tau, Maslow told Sigma Tau officers that: “The technology does not work. If I were you, I would not invest in it.”

258. After Sigma Tau signed a contract in January 2011, with EGT, SKI summarily rejected the contract without justification and without disclosing that it had already entered into the Secret Agreement with Bluebird in accordance with it had been attempting to wrest control of the EGT Vector since 2010.

259. On June 16, 2011, EGT and SKI met at the office of SKI counsel to discuss the resumption of clinical trials.

260. The June 16, 2011 meeting was attended by Pat Girondi and Sam Salman on behalf of EGT and Maslow on behalf of SKI. SKI proposed a new agreement which would give SKI control of the clinical trials and commercial exploitation of the EGT Vector.

261. At the June 16, 2011, meeting Maslow knowingly made false statements to Girondi and Salman to induce EGT to enter into a new agreement with SKI, as set forth in paragraph 74 above.

262. Maslow failed to disclose material facts regarding the Secret Agreement with Bluebird set forth above, including paragraphs 178-190 above.

263. The Secret Agreement and the conspiracy to wrest control of the EGT Vector resulted in the 2011 Agreement, giving SKI control of the development of the EGT Vector.

264. In 2011, EGT was years ahead of Bluebird. Thereafter the conspiracy between SKI and Bluebird allowed Bluebird to catch up to and surpass EGT in the race for a cure for thalassemia and for Orphan Drug Exclusivity. Clinical trials of the EGT Vector were hopelessly delayed, underfunded and ultimately abandoned, leaving Bluebird as the sole company developing a gene therapy for thalassemia and sickle cell diseases.

265. SKI's IND was not filed immediately as promised, but in September 2011, and then was rejected by the FDA as not ready for submission. The IND was finally accepted by the FDA in or about June 2012. The IND was delayed for months by Bluebird by failing to return necessary samples to SKI. The first patient was treated by SKI in November of 2012.

266. In 2012, EGT became aware of reports published in *Oncology Times* and *The New York Times* that Thompson, Agios, and Celgene (a major investor in Agios) had been sued by the University of Pennsylvania for misappropriation of intellectual property developed by Thompson during the time he was scientific director and used it to help start Agios.

267. Based on its concern about the close relationship between Thompson and the principals and primary backers of Bluebird highlighted by these articles, EGT contacted the Board of SKI to address the conflicts of interest and the threat posed to the project.

268. On November 3, 2012, Mr. Girondi sent an email (*Exhibit H*) to the SKI Chairman of the Board Mortimer Zuckerman to express his frustration that his son was waiting for a cure which was being delayed as a result of Dr. Thompson's relationship with Bluebird, stating "Your CEOs' Agios Pharma has received 133 million in raises assisted by the owners of our chief competitor Bluebird who has just abandoned their vector for a new one. This all seems to be following the ending of a bad film. Your CEO is affiliated with our direct competitor and now you're dropping the ball on trials after you grabbed it from us . . . Coincidence. I wouldn't believe it if I was on the jury."

269. On November 6, 2012, Mr. Girondi sent an email to Mr. Zuckerman (*Exhibit I*) stating:

The WSJ published Bluebird as the company for Thalassemia.

Bluebird abandoned their vector. We, SKI-EGT are in Clinical Trials.

Things at SKI are falling behind and too many consequences are pointing to the conflict of interest between your head and Third Rock Partners.

I want my son cured and my investors protected in what is now billions of dollars at risk.

I demanded an emergency meeting. . .

270. EGT sent a letter to the Board of Overseers and Managers of the SKI on November 19, 2002 (*Exhibit J*). EGT expressed its concern that the financial interest of Dr. Thompson in Agios and the financial interest of the significant stakeholders in Agios, was likely to represent a greater incentive to Dr. Thompson than the remuneration from his SKI activities. As the result of the Thompson interconnections and relationships, EGT expressed concern that such conflict would prevent SKI from using its best efforts to commercialize and maintain confidentiality of the EGT Vector.



271. On February 28, 2013, Mr. Girondi sent an email to Mr. Zuckerman (*Exhibit K*) stating that Bluebird has been allowed to catch SKI and EGT in the race for Orphan Drug Exclusivity, and that “Because of Orphan Drug legislation there can be only one winner in the race.” He stated that EGT was prevented from starting clinical trials in 2010 and that he believed that SKI and EGT were both the victims of greed.

272. SKI presented positive data on the clinical trial at the American Society of Hematology meeting in December of 2013. SKI further published the optimal results of its stem cell mobilization trials in ‘Blood’ magazine in March of 2014. The treated patients all underwent gene therapy with no adverse effects. SKI achieved gene expression in the patients and reduced the transfusion regimen in at least one patient. SKI greatly surpassed its expectations.

273. Bluebird has performed clinical trials on at least 30 patients since 2011. SKI has performed clinical trials on four patients. It has not performed any clinical trials in two years.

274. In September 2014, BioMarin Pharmaceutical Inc. approached SKI to commercialize the EGT Vector. Gregg Raskin of SKI told BioMarin that SKI was reticent to negotiate with BioMarin due to preexisting contract rights. Believing that SKI was referring to EGT contract, EGT contacted Eric Cottington at SKI to assure him that EGT was not an impediment to a deal with BioMarin. Mr. Cottington confirmed to SKI that there were no contracts issues that would stop SKI from making a deal with BioMarin.

275. On October 15, 2014, Scott Clarke of BioMarin informed EGT that BioMarin remained interested, but was informed by Gregg Raskin that there was a lien on the technology.

276. On October 20, 2014, Scott Clarke of BioMarin met with SKI and was told that the technology was encumbered and SKI could not move forward with BioMarin. EGT contacted Eric Cottington to inquire whether there was a lien or encumbrance on the technology. Eric Cottington informed EGT that there was no lien or encumbrance.

277. Finally, Scott Clarke was informed by SKI that there was some sort of option on the project and that he should check back in six months. Mr. Clarke informed EGT that based on the manner in which SKI was behaving BioMarin was no longer interested in pursuing the matter. When EGT advised Mr. Cottington of the conversation with BioMarin, he politely asked EGT to mind its own business as they were a professional team that knew exactly what they were doing.

278. On November 24, 2014, the Wall Street Journal announced that BioMarin had agreed to purchase for \$840 million Possensa Holding NV, a pharmaceutical company treating rare diseases with no marketed products.

279. By November 2014, SKI had told EGT and the project supporter, Cooley's International, that SKI had no EGT Vector to continue and would no longer fund the project.

280. In December of 2014, Juno Therapeutics ("Juno") completed its initial public offering. Sadelain was a scientific founder of Juno. SKI received 2,000,000 shares of stock. Sadelain and SKI contributed work on Juno's Chimeric Antigen Receptor Technology (CAR-T) and T-Cell Receptor (TCR) technologies, red-hot areas of immunotherapy, to fight cancer.

281. On January 15, 2015, Cottington told Patrick Girondi that the EGT Vector project was without funding and SKI had no vector remaining to treat patients.

282. In March 2015, Sarepta Therapeutics approached SKI about buying the EGT Vector. Sarepta valued a 50% interest in the project at \$100 million. SKI refused to discuss a transaction without any rationale or justification.

283. In March 2015, an action was commenced by EGT in federal court for replevin of the EGT Vector. Despite having ceased any trials or development of the EGT Vector, ceased all funding of the project and summarily rejecting offers from well-funded pharmaceutical companies, SKI refused to return the EGT Vector to EGT, and was successful in having the replevin claim dismissed.

284. In May of 2015, Sadelain told Girondi: “I’m not supposed to speak with you [but] we could have been ahead of Bluebird...” Sadelain also confirmed that SKI had refused to devote the necessary resources to the development of the EGT Vector: “They have neglected this project... It’s all because of a lack of funding.” Sadelain also admitted that SKI had actually been assisting Bluebird: “We helped Bluebird a lot.” He also said: “We were ahead of BBB [Bluebird] and then they had fifty times the money we had and now they’re ahead of us [though] we have a great vector, a better vector...”

285. On June 29, 2015, Juno announced a \$1 billion investment by Celgene, including purchase of 93 million shares at double the market price. Celgene is a major investor in Bluebird and Agios. In doing so, Celgene reduced its investment in CAR-T cells with Bluebird by \$200 million. A primary competitor to Juno’s CAR-T cell development was eliminated. EGT believes that in exchange for SKI removing EGT as Bluebird’s competition in gene therapy for thalassemia, Bluebird was removed as competition to SKI’s and Juno’s efforts to cure cancer with CAR-T cells.

286. After outstanding results in clinical trials and reporting positive clinical results in peer reviewed journals, SKI has not treated a patient in over two years. The EGT Vector was ground breaking technology with great value to any pharmaceutical company wishing to jump to the forefront of the field of gene therapy. It has been abandoned solely to carry out the partnership and conspiracy of SKI and Bluebird to protect Bluebird’s executives and investors by eliminating the competition to Bluebird and the risk to the \$1.3 billion invested in Bluebird.

287. As Michel Sadelain explained, the EGT Vector has been abandoned by depriving the project of the necessary resources and removing the funding, while Bluebird was speeding ahead in the race to Orphan Drug Exclusivity.

288. Not only was funding withheld, but offers from well-respected pharmaceutical companies – including Sigma Tau, BioMarin and Sarpeta were summarily rejected.

289. Bluebird and SKI engaged in fraudulent conduct in furtherance of the secret agreement between SKI and Bluebird. The overt acts included the conspiracy to fraudulently induce EGT to enter into the 2011 Agreement to eliminate the only competition to Bluebird, and the ultimate abandonment of the EGT Vector.

290. EGT was damaged as the result of that conspiracy.

291. EGT is entitled to damages in the amount to be proven at trial, plus punitive damages in an amount necessary to deter future misconduct by Defendant.

**FIFTH CAUSE OF ACTION**  
**Unfair Competition Against Bluebird**

292. Plaintiff repeats and realleges each and every allegation contained in paragraphs 1 through 291 as if fully set forth herein.

293. Bluebird was aware that the EGT Vector of its competitor, EGT, was better than its own product. Bluebird also knew that it could not obtain the EGT's rights in the EGT Vector by negotiating with EGT since it had been rejected by EGT in June 2010. Accordingly, it entered into the Secret Agreement with SKI in 2010 to halt clinical trials and fraudulently induce EGT to transfer the EGT Vector and EGT Intellectual Property to SKI.

294. Under the Secret Agreement, the EGT Intellectual Property was transferred by SKI to Bluebird for free to allow Bluebird to develop a safe vector. Such transfers occurred during secret meetings in 2010 and 2011 prior to the June 2011 Agreement between SKI and EGT.

295. Bluebird had the ability to incorporate all of EGT's Intellectual Property into the Bluebird vector.

296. Bluebird received the free services of SKI scientists as consultants to develop a safe Bluebird vector using the EGT Intellectual Property.

297. The Secret Agreement provided that SKI would be totally reliant on Bluebird for funding of clinical trials and manufacture of vector, while providing that Bluebird had absolutely no obligation to provide the funding.

298. Bluebird provided no funding for clinical trials of the EGT Vector while under the terms of the Secret Agreement, SKI was precluded from obtaining funding from any other commercial source.

299. Under the Secret Agreement, EGT's clinical trials were halted, EGT was defrauded into signing its Intellectual Property to SKI, it was used to develop the Bluebird vector and the EGT Vector was abandoned.

300. Bluebird engaged in such conduct not out of any effort to develop a better drug, but instead to destroy the development of the EGT Vector and eliminate EGT as a competitor so Bluebird and to enable Bluebird to receive Orphan Drug Exclusivity.

301. Bluebird acted with malice and bad faith against EGT, SKI and patients suffering from this fatal disorder.

302. Bluebird developed its own vector, incorporating the EGT Intellectual Property and palmed off the vector as its own product.

303. Bluebird's conduct constitutes unfair competition.

304. EGT was damaged as the result of that unfair competition.

305. EGT is entitled to damages in the amount to be proven at trial, plus punitive damages in an amount necessary to deter future misconduct by Defendant.

**SIXTH CAUSE OF ACTION  
Injunctive Relief Against Bluebird**

306. Plaintiffs repeat and reallege the allegations contained in paragraphs 1 through 305.

307. By the improper actions of Bluebird which constitute conspiracy to defraud and unfair competition, as described above, Bluebird has substantially interfered, and continues to substantially interfere, with Plaintiff's right: (1) to develop the EGT Intellectual Property, (2) receive Orphan Drug Exclusivity and (3) to use the EGT Intellectual Property in connection with any medical treatment or drug for diseases including hemoglobinopathies such as Thalassemia.

308. If Bluebird is allowed to continue its improper and illegal actions, Plaintiff will suffer irreparable harm.

309. Plaintiff has no adequate remedy at law.

310. The balance of the equities favor Plaintiff in that Plaintiff seeks injunctive relief only to the extent necessary to remedy Bluebird's: (i) misappropriation of the EGT Intellectual Property to incorporate it into its own medical treatments or drugs; (ii) efforts to permanently hinder the development of the EGT Vector; and (iii) eliminate EGT as a competitor.

311. By reason of the foregoing, Plaintiff is entitled to a permanent injunction restraining and enjoining Bluebird and its agents, servants, employees, representatives and persons acting in concert or participation with them from using any EGT Intellectual Property in connection with the sale, licensing or commercial exploitation any medical treatment or drug.

**SEVENTH CAUSE OF ACTION  
Unjust Enrichment Against Bluebird**

312. Plaintiff repeats and realleges each and every allegation contained in paragraphs 1 through 311 hereof as if fully set forth herein.

313. Defendant Bluebird has been unjustly enriched by having misappropriated the EGT Intellectual Property and know-how and all results from clinical trials of the EGT Vector without paying any compensation.

314. It is against equity and good conscience to permit Bluebird to retain the EGT Intellectual Property and know-how and all results from clinical trials of the EGT Vector and to use same in connection with sale, licensing or commercial any medical treatment or drug without compensating EGT.

315. Plaintiff is entitled to damages from Bluebird in an amount to be determined at trial and is entitled to punitive damages to deter future misconduct because, as set forth above, Defendant's conduct was willfully, wantonly and maliciously designed to benefit themselves at the expense and diminishment of Plaintiff and the general public.

**WHEREFORE**, Plaintiff respectfully requests that this Court grant the following relief:

On the First Cause of Action, enter judgment in an amount to be proven at trial, plus punitive damages in an amount necessary to deter future misconduct by Defendant SKI;

On the Second Cause of Action, enter judgment in an amount to be proven at trial against Defendant SKI;

On the Third Cause of Action, enter judgment in an amount to be proven at trial against Defendant SKI;

On the Fourth Cause of Action, enter judgment in an amount to be proven at trial against Defendants SKI and Bluebird, plus punitive damages in an amount sufficient to deter future misconduct by Defendants SKI and Bluebird;

On the Fifth Cause of Action, enter judgment in an amount to be proven at trial against Defendant Bluebird, plus punitive damages in an amount necessary to deter future misconduct by Defendant Bluebird;

On the Sixth Cause of Action, granting an injunction restraining and enjoining Bluebird and its agents, servants, employees, representatives and persons acting in concert or participation with them from using any of the EGT Intellectual Property in connection with the sale, licensing or commercial use of any medical treatment or drug; and

On the Seventh Cause of Action, enter judgment in an amount to be proven at trial against Defendant Bluebird, plus punitive damages in an amount necessary to deter future misconduct by Defendant Bluebird.

Awarding such other damages and relief as the Court deems appropriate.



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*Attorneys for Errant Gene Therapeutics, LLC*



# Exhibit E

COMMONWEALTH OF MASSACHUSETTS

SUFFOLK, SS

SUPERIOR COURT DEPARTMENT  
OF THE TRIAL COURT

ERRANT GENE THERAPEUTICS, LLC,

Plaintiff,

v.

THIRD ROCK VENTURES, LLC and  
NICK LESCHLY,

Defendants.

Civil Action No. 19-1832

**AMENDED COMPLAINT AND JURY DEMAND**

Plaintiff Errant Gene Therapeutics, LLC ("EGT") brings this action against Defendants Third Rock Ventures, LLC ("TRV") and Nick Leschly ("Leschly") (TRV and Leschly together, "Defendants") for tortious interference, misappropriation of trade secrets, civil conspiracy, unjust enrichment, and violations of Massachusetts General Laws ch. 93A.

**INTRODUCTION**

1. Defendants are the venture capital financiers of a multibillion-dollar biotech company named Bluebird Bio, Inc. ("BBB"). Beginning in 2010, Defendants maliciously embarked upon a secret and unlawful scheme to misappropriate highly confidential intellectual property concerning a potentially life-saving gene therapy developed by EGT, a small biomedical company. In furtherance of their scheme, and with improper and unlawful motives, Defendants converted and exploited EGT's proprietary technology, destroyed and effectively put EGT (BBB's only competitor) out of business, and enriched themselves at the expense of EGT and patients by,

SUFFOLK SUPERIOR COURT  
CIVIL CLERK'S OFFICE  
2019 SEP 25 PM 4:01  
MICHAEL JOSEPH DUNNAN  
CLERK / MAGISTRATE

among other things, using their newfound monopoly power to charge unconscionably high prices for treatment of a crippling disease.

2. Keeping their brazen scheme hidden was vital to the success of Defendants' willful plan to "shut down" EGT. Defendants actively concealed their bad faith conduct from EGT through various improper means and unlawful acts, including, without limitation, financially inducing EGT's research partner, Sloan Kettering Institute for Cancer Research ("SKI"), to disclose confidential information to BBB; financially inducing SKI to enter into secret, undisclosed agreements with BBB; making material misstatements and omissions (including in public filings) to conceal Defendants' wrongdoing; and lulling EGT into inaction by feigning interest in EGT as a business partner. It was not until EGT filed a lawsuit in New York and documents were produced in discovery in June 2016 that EGT began to uncover the nature and scope of Defendants' fraudulent scheme.<sup>1</sup>

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<sup>1</sup> As described in greater detail herein, Defendants actively concealed their fraudulent scheme from EGT for years. It was only when EGT obtained documents during discovery in June 2016 after filing a lawsuit against SKI in New York that EGT began to understand the nature and extent of BBB's prominent role in the fraud. Even then, however – and in furtherance of their ongoing attempt to hide their misconduct – SKI and non-party TRV designated virtually every document produced in June 2016 as Attorneys' Eyes Only ("AEO") under a protective order. The AEO designation provided that EGT's counsel was prohibited from sharing the documents with EGT or using the documents for any purpose other than in connection with the New York action. After reviewing the documents produced by SKI and TRV, EGT filed a new complaint in January 2017 in New York naming both SKI and BBB as defendants. When BBB finally produced documents in November 2018, the documents demonstrated how extensively BBB, Leschly, and TRV conspired with SKI to misappropriate EGT's intellectual property and to put EGT out of business. Yet again, however, BBB designated nearly all of those documents – including an e-mail in which Leschly instructs a BBB employee to "shut down" EGT – as confidential. After the November 2018 production of documents, EGT requested leave of the New York court to file an amended complaint adding Leschly and TRV as defendants. The court denied EGT's request, but acknowledged that EGT was free to initiate a separate action in Massachusetts against Leschly and TRV. Only very recently has EGT been permitted to use certain of these "confidential" documents that Defendants sought to shroud in secrecy for years. On August 26, 2019 and September 23, 2019, the New York court issued orders directing that more than 100 documents filed under seal

3. Since the time Defendants started secretly using EGT's intellectual property to build its business – and after eliminating EGT as a competitor – the value of Defendants' interests in BBB has increased by untold millions of dollars (as BBB's market cap has ballooned from less than \$1 billion to nearly \$6 billion). Moreover, since 2014, Leschly has sold more than \$80 million worth of shares, and has earned more than \$55 million in total compensation from BBB (almost \$24 million in 2018 alone).

4. Although the full extent of the damages to EGT is not readily calculable outside of trial, the direct damages known to EGT are at least in the hundreds of millions of dollars.

### **THE PARTIES**

5. Plaintiff Errant Gene Therapeutics, LLC is a Delaware limited liability company with a principal place of business located at 218 North Jefferson Street, Chicago, Illinois.

6. Upon information and belief, Defendant Third Rock Ventures, LLC is a Delaware limited liability company with a principal place of business located at 29 Newbury Street, Boston, Massachusetts. At all relevant times, TRV orchestrated, directed, and/or participated in BBB's conduct set forth herein. In addition, TRV actively participated in and orchestrated the unlawful conduct of SKI set forth herein.

7. Upon information and belief, Defendant Nick Leschly is an individual who resides at 68 School Street, Weston, Massachusetts. At all relevant times, Leschly was Chief Executive Officer of BBB and orchestrated, directed, and/or participated in BBB's conduct set forth herein. In addition, Leschly actively participated in and orchestrated the unlawful conduct of SKI set forth herein.

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by Defendants and hidden from public view be made immediately available to EGT and the public. These documents conclusively demonstrate Defendants' unlawful acts.

### **JURISDICTION AND VENUE**

8. Jurisdiction for this action is proper pursuant to G.L. c. 93A, §11 and G.L. c. 212, §3.
9. Personal jurisdiction over the Defendants is proper under G.L. c. 223A, §§ 2, 3.
10. Venue in Suffolk County Superior Court is proper under G.L. c. 223, §8.

### **FACTS**

#### **Sickle Cell Disease and Thalassemia**

11. Sickle cell disease and thalassemia are rare genetic blood disorders. They are caused by errors in the genes for hemoglobin, the protein in red blood cells that carries oxygen throughout the body. Both sickle cell disease and thalassemia are characterized by chronic anemia, pain, and complications due to organ and tissue damage.

12. Sickle cell disease is common among those whose ancestors came from sub-Saharan Africa. Individuals suffering from sickle cell disease rarely live beyond their late 40s.

13. Thalassemia is rare in the United States, but is more common in the Mediterranean. On average, thalassemia patients rarely survive beyond their late 20s.

#### **Rocco Girondi Is Diagnosed with Thalassemia**

14. In 1992, Rocco Girondi was diagnosed with thalassemia. Rocco was two years old when he was diagnosed with the disease.

15. Rocco has a severe form of thalassemia. Since he was diagnosed, Rocco has undergone blood transfusions every three to four weeks. He takes 10 pills each day to reduce the toxic iron that builds up in his blood and organs as a result of the disease.

#### **Patrick Girondi, Rocco's Father, Founds EGT**

16. In 1993, a year after Rocco was diagnosed with thalassemia, Rocco's father, Patrick Girondi, left a successful career at the Chicago Board of Trade to start a pharmaceutical company with the goal of curing thalassemia. That year, Girondi formed Emerging Pharmaceutical Technologies, which eventually became EGT.

17. EGT entered into partnerships with world-renowned medical centers and hospitals, such as SKI, the National Institute of Health ("NIH"), and the World Health Organization, and raised tens of millions of dollars for sickle cell disease and thalassemia research. EGT's goal is to develop a life-saving gene therapy for sickle cell disease and thalassemia.

#### **EGT's Partnership with SKI**

18. In 2000, EGT contacted Dr. Michel Sadelain, a world-renowned researcher at SKI. Sadelain had published a paper describing his experiments with gene therapy for thalassemia. In his paper, Sadelain reported that he had successfully cured five generations of thalassemic mice.

19. Gene therapy involves inserting a healthy gene into the stem cells of the body to correct a defective gene. In 2000, gene therapy was largely considered unsafe, in part due to the 1999 death of a patient receiving gene therapy treatment. Nevertheless, EGT saw potential in Sadelain's gene therapy for thalassemia, and began funding Sadelain's research.

20. In 2003, Sadelain advised EGT that SKI had decided that it would no longer support Sadelain's gene therapy research. EGT attempted to interest other pharmaceutical companies in Sadelain's research but these efforts were unsuccessful, largely because of the stigma at that time surrounding gene therapy.

21. In 2005, with no other pharmaceutical companies interested in Sadelain's drug therapy, EGT purchased from SKI the exclusive, worldwide license and right to exploit Sadelain's drug therapy for use in blood disorders, including in sickle cell disease and thalassemia. Without

EGT's acquisition of the rights to the drug therapy, Sadelain's research would have ended. EGT's purchase of these rights was memorialized in a license agreement between EGT and SKI dated March 7, 2005 (the "2005 Agreement").

#### **EGT Develops the EGT Vector**

22. From 2005 to 2009, EGT, with the assistance of Sadelain, and in partnership with SKI and other research institutions, developed a revolutionary gene therapy to treat thalassemia in accordance with the highest manufacturing and testing standards. EGT named its gene therapy treatment "Thalagen."

23. EGT's therapy involves inserting new genetic material into affected cells of a patient through a targeted delivery system, known as a "vector." The vector delivery system, which is a de-activated HIV virus, is a critical component of EGT's technology, and greatly affects the efficacy, safety, and commercial viability of its therapy. The innovative manner in which EGT's vector is manufactured is a defining feature of EGT's gene therapy treatment for thalassemia (the "EGT Vector").

24. EGT worked closely with SKI and Sadelain to develop and improve the EGT Vector. EGT funded multiple scientific contracts with SKI and solicited SKI's participation in major decisions regarding the therapy, such as the timing of applications for regulatory approval to treat patients in clinical trials.

25. Between 2006 and 2009, EGT achieved several significant milestones with respect to its development of the EGT Vector.

26. In January 2006, the Food and Drug Administration (the "FDA") granted EGT an "orphan drug designation" for its vector. An "orphan disease" is a disease, such as thalassemia, which affects a small percentage of the population. Assigning "orphan" status to a disease is

intended to increase investment in research to treat medical conditions which, because they are so rare, would not be profitable to produce without government assistance. One benefit of obtaining an orphan drug designation is that it leads to market exclusivity. Once a company is awarded orphan drug exclusivity, the FDA is generally barred from approving any other orphan drug designation for the same orphan disease for seven years.

27. In 2007, NIH's Recombinant DNA Advisory Committee unanimously approved the EGT Vector. At that time, such committee approval was a necessary hurdle for all gene therapies.

28. In 2008, EGT completed the Pre-IND (Investigational New Drug) Consultation Program with the FDA. The IND program is the means by which a pharmaceutical company obtains permission to start human clinical trials and to ship an experimental drug across state lines.

29. In 2009, EGT was awarded orphan drug designation in Europe by the European Medicines Agency (the "EMA"), the European equivalent of the FDA. Once a company is awarded orphan drug exclusivity by the EMA, the EMA is generally barred from approving any other orphan drug designations for the same orphan disease for ten years. Also in 2009, the United States Patent and Trademark Office issued a patent to Sadelain and other inventors of the EGT Vector.

30. As a result of its diligent and pioneering work to find a treatment for thalassemia, EGT received widespread recognition in its field, including awards from the World Health Organization and various thalassemia foundations.

31. More importantly, by 2010, EGT had transformed the EGT Vector from a technology with little commercial interest into viable treatment for individuals suffering from thalassemia. As a result, the EGT Vector and associated know-how was worth at least tens of



millions of dollars and, upon the successful completion of a credible clinical program demonstrating safety in humans, hundreds of millions of dollars.

#### **Measures Taken by EGT to Guard Its Proprietary Information**

32. At all times relevant, EGT has taken care to protect and guard the secrecy of its clinical data, know-how, and other trade secrets, including the EGT Vector. Among other things, EGT enters into confidential disclosure agreements (“CDAs”) or other confidentiality agreements with all outside partners and vendors with access to EGT’s confidential information; carefully restricts access to its facilities; keeps sensitive information in locked cabinets and offices; secures its computers with passwords and the latest patches; hosts its e-mail and web servers on a private, secure platform maintained with the highest security standards; requires a private key to make modifications to electronic files containing highly sensitive information; and takes measures to mark documents containing trade secrets or other sensitive or proprietary information as “Confidential.”

#### **Genetix Pharmaceuticals, Inc.**

33. Upon information and belief, Genetix Pharmaceuticals, Inc. was founded in 1992 as a biotechnology company focused on developing gene therapies. Genetix (now BBB) is located in Cambridge, Massachusetts.

34. Using technology invented by Dr. Philippe Leboulch, Genetix developed a gene therapy drug intended to treat thalassemia. Like the EGT Vector, Genetix’s gene therapy relied on a vector delivery system (the “Genetix Vector”).

35. In or around May 2009, the Genetix Vector caused a thalassemia patient in France to develop a condition known as “clonal dominance,” which is a precursor to cancer. In response,

in June 2009, both the French government and the FDA banned all gene therapy trials for several months.

36. As the only two companies with a serious drug therapy treatment for thalassemia, Genetix (now BBB) and EGT are direct competitors. As of 2009, when the Genetix Vector was blamed for causing clonal dominance in a thalassemic patient, the EGT Vector was years ahead of the Genetix Vector in terms of development. The EGT Vector was more effective, less expensive, and more patient-friendly than the Genetix Vector. The successful development of the EGT Vector was the direct result of EGT's substantial investment in improving and commercializing its gene therapy for thalassemia.

37. As SKI's Sadelain would later explain in an e-mail comparing the EGT and Genetix Vectors:

We've just spent 2 years improving the manufacturing. We made enough vector for 10 patients in one production run. [Genetix] makes one batch at the time for one patient. That is not viable. Our process is. Their vector has an unstable structure (it "rearranges," as found in their second patient). That makes it very unlikely that it will ever be commercialized, at least with its current sequence. Our vector is structurally very stable. Based on published mouse studies, our vector expresses better than theirs.

Indeed, in 2010, in view of the deficiencies plaguing the Genetix Vector, which Sadelain characterized as a "lemon," Sadelain was "baffl[ed] that [BBB] would even continue with [its] vector."

### **Third Rock Ventures**

38. Defendant Third Rock Ventures is a Massachusetts-based venture capital firm. TRV was founded in 2007. Today, TRV's portfolio includes more than 50 companies.

39. Defendant Nick Leschly was a founding partner of TRV. Leschly played an integral role in the overall formation, development, and business strategy of several of TRV's

portfolio companies, including BBB and a large pharmaceutical company named Agios Pharmaceuticals, Inc.

#### **Leschly and TRV Contact SKI to Learn About EGT's Gene Therapy**

40. In or around September 2009, TRV partners Leschly and Dr. Philip Reilly contacted SKI's Sadelain to express TRV's growing interest in the genetic treatment of orphan disorders, including thalassemia. Leschly and Reilly requested to meet with Sadelain to discuss SKI's plans for these genetic treatments.

41. In response to Leschly and Reilly's request, Sadelain advised Leschly and Reilly in an e-mail dated October 5, 2009, that it would be "more appropriate" for Leschly and Reilly "to meet with EGT, to whom we have licensed our globin-related technology and with whom we are planning clinical trials in the US and Europe."

42. A few days later, Sadelain introduced Leschly and Reilly to Sam Salman, EGT's President, over e-mail. In his introductory e-mail dated October 8, 2009, Sadelain reiterated that "[s]ince [SKI] has licensed our globin vector technology to EGT, Dr. Viviane Martin, who heads [SKI]'s Office of Industrial Affairs, has recommended that Third Rock Ventures directly contact Sam Salman, President of EGT. You are now in contact!"

43. Accordingly, as of October 2009, Leschly and TRV had been advised in writing on two separate occasions that SKI had licensed its gene therapy for sickle cell disease and thalassemia to EGT and, consequently, that TRV should communicate with EGT with respect to TRV's interest in EGT's gene therapy for thalassemia.

#### **TRV and EGT Meet in Boston**

44. On or around October 19, 2009, Leschly and Reilly met with Salman and an EGT advisor at TRV's headquarters in Boston. During the meeting, Leschly expressed TRV's desire

to develop a “platform” for the development of gene therapies for thalassemia and sickle cell disease. He advised EGT that TRV was considering an investment in the gene therapy invented by Sadelain (and under development by EGT) and/or the competing gene therapy invented by Leboulch (and under development by Genetix).

45. Salman explained that EGT welcomed the opportunity to partner with TRV. He also asked Leschly why TRV was considering an investment in Leboulch’s gene therapy, which had proven unsuccessful (and led to an FDA ban of gene therapy treatment). Salman explained to Leschly the reasons he believed that the EGT Vector had the greatest likelihood of commercial success. Leschly recognized certain limitations of the Genetix Vector, and acknowledged that the EGT Vector invented by Sadelain was superior to the Genetix Vector.

46. In an e-mail to Leschly after the October 19, 2009 meeting, Salman reiterated EGT’s interest in partnering with TRV, but emphasized that, “as discussed during our meeting, our science cannot be compromised.”

#### **TRV Invests \$35 Million in Genetix and Installs Leschly as CEO**

47. In March 2010, TRV invested \$35 million in Genetix. In September 2010, TRV partner Leschly was installed as the new Chief Executive Officer of Genetix, and TRV partner Reilly was named Chief Medical Officer. At the same time, TRV changed Genetix’s name to Bluebird Bio, Inc. Upon information and belief, TRV changed Genetix’s name to distance the company from the 2009 ban on gene therapy caused by the Genetix Vector.

#### **TRV and Leschly’s “Apollo 13 Moment”**

48. After TRV finalized its \$35 million investment in Genetix, Leschly “got bad news,” according to an interview Leschly gave to the *New York Times*, published in November 2017. The manufacturer of the Genetix Vector advised Leschly that it would cost \$1 million to create enough

of the Genetix Vector to treat a single patient. “It was an Apollo 13 moment,” Leschly said. “Everything at the company ha[d] stopped.”

49. Upon information and belief, it was at or around the time of this “Apollo 13 moment” that TRV and Leschly determined that access to EGT’s technology was essential in overcoming the problems plaguing the Genetix Vector.

#### **TRV Again Contacts SKI to Inquire about EGT’s Technology; SKI Expresses Concern**

50. In or around May 2010, TRV arranged a meeting with Sadelain at SKI to learn more about Sadelain’s “progress and plans” for the EGT Vector. Sadelain forwarded TRV’s proposed meeting agenda to Vivianne Martin and Andrew Maslow, both members of SKI’s Office of Industrial Affairs. On May 3, 2010, Martin responded to TRV’s request for a meeting with Sadelain as follows:

Michel [Sadelain], I don’t quite see how 3rd Rock/Genetix can make the best out of MSK/Genetix technology *w/o letting one sit on a shelf*. Also they know that we entered into a license with EGT, and they are coming to you, not to EGT when they perfectly know that they should talk to EGT to get rights to the technology. *I honestly do not see what they are seeking . . . [sic] besides them doing competitive intelligence . . . .* (emphasis added).

51. In response to Martin, Sadelain acknowledged that Martin “may well be right,” and advised that he will ask TRV for some clarification during their meeting that morning. Martin responded promptly with the same unguarded skepticism: “I don’t know. . . *I just cannot see anything else that makes sense*. . . [sic] nor does Andy [Maslow] BTW” (emphasis added).

52. After his meeting with Leschly and TRV, Sadelain e-mailed Martin to advise her that TRV and Leschly had separately met that day with a former EGT investor. Sadelain, assuming (correctly) that EGT was unaware of TRV’s meeting with the EGT investor, explained “Quelle soupe!”

### **EGT Rejects TRV's Suggested Collaboration Unless TRV Guarantees Use of EGT Vector**

53. Shortly thereafter, TRV contacted EGT to arrange a meeting at Genetix's headquarters in Cambridge. The meeting between EGT and "the Genetix/3rd Rock Team" (the term Genetix used to describe its team) was scheduled for June 8, 2010.

54. On June 8, EGT President Salman and EGT founder Girondi met at Genetix's Cambridge headquarters with Leschly, TRV's Neil Exter, and Genetix's Chief Scientific Officer, Mitch Finer, to discuss TRV's potential partnership with EGT. TRV made it clear during the meeting that it was only willing to collaborate with EGT if EGT were willing to accept compensation that represented a fraction of the value of the EGT Vector.

55. Girondi noted that the Genetix Vector had proven to be unsafe and expressed his view that it was inferior to the EGT Vector. He also advised TRV that EGT would not agree to sell EGT's intellectual property, including the EGT Vector, unless TRV assured EGT that it would develop the EGT Vector.

56. In e-mail correspondence between Girondi and SKI two days after EGT's meeting with TRV, SKI advised Girondi that SKI was "lucky to have you on our team," commended EGT for years of effort to develop the EGT Vector, and expressed SKI's conviction that TRV's apparent decision to focus on the Genetix Vector is "Third Rock's loss."

### **Defendants Embark Upon Scheme to Eliminate Only Competitor and Obtain EGT Vector**

57. Unable to acquire the EGT Vector from EGT on TRV's terms – and increasingly concerned that it had invested \$35 million in an inferior gene therapy – Defendants entered into and engaged in a conspiracy and partnership with SKI to block clinical trials for the EGT Vector, wrongfully wrest control of the EGT Vector from EGT, and remove EGT from competition for orphan drug exclusivity. Defendants concocted their unlawful scheme in Massachusetts – and

carried out their unfair and deceptive acts in furtherance of their scheme from Massachusetts – where both TRV and Leschly are engaged in business. TRV and Leschly actively participated in and directed SKI’s dealings with EGT in furtherance of the conspiracy.

58. On June 18, 2010, Leschly sent an e-mail to Mitch Finer at Genetix referencing Girondi and expressing concern that TRV and Genetix “*need to shut him [i.e., Mr. Girondi] down*” (emphasis added). Finer responded that he “*want[s] to get [Girondi] to buy into a CDA to review Michel [Sadelain]’s data*” (emphasis added). To convince Girondi to sign a CDA, Finer implored Leschly to “*Be nice, suck up, etc. . . [sic] if you think (and I think) that [Sadelain] has valuable data*” (emphasis added).

59. In August 2010, Dr. Craig B. Thompson was named Chief Executive Officer of SKI. Thompson has a deep, long-standing relationship with Leschly, TRV, and BBB. Among other things, TRV had invested more than \$33 million in Agios Pharmaceuticals, the company founded by Thompson, and Leschly had served as the Chief Business Officer for Agios in 2009-2010. Defendants seized upon Thompson’s appointment as CEO of SKI to obtain SKI’s assistance to “shut down” EGT – BBB’s only competitor.

#### **TRV, Leschly, and BBB Arrange for “Technical Presentation” from Sadelain**

60. In October 2010, TRV, Leschly, and BBB arranged a November 2, 2010 meeting with SKI. According to the proposed agenda for the meeting, Sadelain was scheduled to make a “Technical Presentation” to Leschly and others at TRV and BBB that would last almost two hours. EGT was never advised of the November 2, 2010 meeting or that technical data concerning its intellectual property would be disclosed to its only competitor.

61. On November 2, 2010, the same day of the secret meeting, BBB and SKI executed a CDA (the “2010 CDA”). Leschly signed the 2010 CDA on behalf of BBB. Neither the existence nor the contents of the 2010 CDA was disclosed to EGT.

62. The 2010 CDA provides that SKI would be disclosing to BBB “trade secrets, know-how, inventions, technical data or specifications, testing methods . . . research and development activities, control and inspection practices, [and] manufacturing processes and methods.” It also provides as follows:

Neither party to this Agreement shall reveal the fact that Confidential Information has been disclosed pursuant to this Agreement, *nor that either party is conducting or has conducted discussions or negotiations in furtherance of a business relationship with the other* (emphasis added).

This agreed-upon prohibition on disclosure of the discussions between SKI and Defendants “in furtherance of a business relationship with the other” memorialized that the dealings between SKI and Defendants would be concealed. The concealment of these discussions from EGT was critical to the success of Defendants’ fraudulent scheme.

63. In view of the 2010 CDA, EGT can only infer that Sadelain presented confidential trade secrets and know-how about the EGT Vector to TRV, Leschly, and BBB during the November 2, 2010 meeting.

**BBB Board Explicitly Discusses “Eliminat[ing]” EGT by Partnering with SKI; SKI Committee Discusses Terminating EGT License**

64. During a November 2010 presentation to BBB’s Board of Directors – which Leschly chaired – BBB specifically discussed the “pros/cons” for a BBB partnership with SKI. One of the “pros” identified by BBB was that a partnership with SKI “eliminates [BBB’s] most threatening competitor,” EGT. BBB also compared the EGT Vector with the Genetix Vector, acknowledging that:



- the EGT Vector has a “[s]ignificantly increased probability of success” in certain thalassemia patients;
- the EGT Vector has a “3-5x improvement in per copy expression vs. BBB vector”; and
- the EGT Vector will “Increase probability and magnitude of success” and result in a “Substantial increase in expected value/ROI.”

65. In parallel with plans to “eliminate” EGT hatched by BBB, SKI’s Ad Hoc Committee on Technology Transfer met on December 7, 2010 to discuss terminating its relationship with EGT. Prior to this meeting, and in furtherance of their conspiracy, Defendants had induced SKI to initiate action to terminate its contract with EGT. During the December 7, 2010 meeting, SKI’s committee discussed the efforts that were already underway to terminate the 2005 Agreement with EGT. The committee also discussed negotiating a license with BBB once EGT’s license had been terminated.

**TRV and BBB Meet with Sadelain and, the Next Day, Enter into Secret Letter Agreement**

66. On February 16, 2011, TRV and BBB met with Sadelain and his research partner at SKI, Dr. Isabelle Riviere. EGT was never made aware of this secret meeting.

67. The next day, February 17, 2011, BBB and SKI entered into a Letter of Intent. The “Letter of Intent,” which is signed by Leschly, confirms BBB’s interest in EGT’s proprietary intellectual property and acknowledges that the “rights” with respect to the EGT Vector are “the subject of a license agreement between [SKI] and a third party.” Leschly insists in the Letter of Intent that SKI provide BBB with exclusive rights to negotiate to acquire the EGT Vector. The existence of the Letter of Intent was never disclosed to EGT.

### **SKI Deletes Reference to Terminating EGT from Board Minutes**

68. In a March 29, 2011 meeting of SKI's Ad Hoc Committee on Technology Transfer, attended by SKI's Thompson, among others, the committee voted to delete from the minutes of its December 7, 2010 meeting a reference to terminating the 2005 Agreement with EGT. The March 29, 2011 committee meeting minutes, which confirm the active concealment in connection with Defendants' scheme, state as follows:

The minutes of the meeting of December 7, 2010 were approved with one correction. The following sentence was deleted: "Efforts are underway to terminate the license with EGT, and once completed, negotiate a license with Bluebird bio."

### **TRV, Leschly, and BBB Meet Again with SKI**

69. On June 6, 2011, TRV and BBB met again with Sadelain and Riviere at BBB's headquarters in Cambridge. This time, Leschly and others at BBB and TRV dedicated an entire day to meeting with SKI. Like previous meetings, the June 6, 2011 meeting was kept secret from EGT.

70. According to the agenda circulated in advance of the June 6, 2011 meeting, Leschly and his team were scheduled to meet for two hours before Sadelain and Riviere even arrived so that they could discuss what, exactly, they "want from this relationship." Then, Sadelain and Riviere were scheduled to present highly proprietary technical information about the EGT Vector and related know-how for two full hours – all in violation of the 2005 Agreement – after which the parties were scheduled to discuss "areas of collaboration." The agenda items listed for discussion with Sadelain and Riviere are a succinct list of the most critical and sensitive information and trade secrets concerning the EGT Vector: (1) the development of the EGT Vector, from the beginning of the EGT Vector's development, all the way up to "the final clinical construct"; (2) the "titration methodology" used for the EGT Vector; (3) "pharmacology studies

demonstrating therapeutic correction”; (4) the “results of transduction engineering runs”; and (5) the “status of regulatory filings” in connection with EGT’s Vector.

71. Recently disclosed contemporaneous notes taken by Mitch Finer of BBB during the June 6, 2011 meeting reveal the extent to which SKI disclosed EGT’s technology and confidential trade secrets to Leschly, TRV, and BBB. Finer’s notes confirm that Sadelain and Riviere shared highly proprietary information, developed over many years by EGT, including the type of “enhancers” used in the EGT Vector, the “promoter length,” and a comprehensive explanation of what worked with respect to the development, engineering, and manufacturing of the EGT Vector.

72. Like many other documents referred to herein, both the June 6, 2011 meeting agenda and Finer’s comprehensive notes from the meeting were only made available for use by EGT in late August 2019.

#### **SKI Induces EGT to Terminate 2005 License Agreement**

73. On June 16, 2011, EGT and SKI met to discuss the status of future clinical trials for the EGT Vector. During the meeting, SKI, with Leschly and TRV’s involvement, direction, and participation, proposed that EGT and SKI enter into a new agreement whereby EGT would grant SKI control over both the clinical trials and the commercial exploitation of the EGT Vector.

74. At no time during the June 16, 2011 meeting did SKI disclose that it had executed the 2010 CDA with BBB or the Letter of Intent with BBB dated February 17, 2011 (nor could SKI do so under the terms of the 2010 CDA). Nor did SKI disclose that it had secretly met with Leschly, TRV, and BBB on multiple occasions prior to June 16, 2011 to negotiate a proposed business relationship, including on May 3, 2010 to discuss Sadelain’s “progress and plans” for treatment of Thalassemia, on November 2, 2010 during which Sadelain gave a “technical presentation” that lasted nearly two hours, on February 16, 2011, and on June 6, 2011 for an entire

afternoon and during which extensive, highly proprietary and confidential information was shared with Leschly, BBB, and TRV, all in violation of the 2005 Agreement.

75. In a further effort to induce EGT to enter into a new agreement with SKI, and with the knowledge and/or participation of Defendants, SKI's Maslow knowingly made multiple false statements to EGT's Gironi and Salman at the June 16, 2011 meeting. Maslow stated, for example, that "SKI had spent \$1.5 million to write the IND, and that the IND is done and ready to be filed [with the FDA] immediately." Maslow repeated these statements several times, even though he knew that the IND application was not ready to be filed. Maslow further stated that the first patient would be treated no later than October 2011, even though he knew that timeline would not be met. Finally, Maslow also stated that SKI would conduct clinical trials "then license the technology to the highest bidder." Upon information and belief, Maslow made these misrepresentations with the knowledge and approval of Defendants and in furtherance of Defendants' wrongful scheme. Based on these representations, EGT entered into the agreement believing that giving SKI control over the clinical trials was likely to result in the quickest path to the commencement of clinical trials.

76. In reliance upon these material misrepresentations and omissions, EGT entered into an agreement with SKI on June 17, 2011 (the "2011 Agreement") which terminated the 2005 Agreement and granted SKI all rights to develop the EGT Vector. Under the agreement, EGT assigned to SKI all of its "right, title, and interest" in the EGT Vector and related intellectual property for treating sickle cell disease and thalassemia. In exchange, SKI agreed to assume the right and obligation to commercially develop the EGT Vector, to fund and conduct clinical trials, and to pay EGT 50% of all revenue generated from the EGT Vector.

77. Incredibly, after obtaining discovery in the New York action, EGT learned that BBB and Defendants were intimately involved in reviewing drafts of the 2011 Agreement and actually *participated* in negotiating it. A June 19, 2011 e-mail from BBB's Senior Director of Business Development, Cyrus Mozayeni, to Leschly and others at BBB with the subject line "MSK-EGT license terminated!" states as follows:

On Friday evening I witnessed the grand finale to the ongoing fireworks display between MSK and EGT . . . and it was truly spectacular! In fact it was an unbelievable roller coaster the entire week. I can fill you in on the details later (at some point I may even write a screen play for S. Spielberg . . . hopefully not S. King). But the bottom line is that it ended better than I could have hoped. The license agreement is officially terminated and MSK has agreed to give them 50% of any future deal they could do on that specific vector . . . . As compared to the earlier version of the settlement agreement, the current one enables a more straightforward deal structure for us . . . . (emphasis in original).

This e-mail demonstrates that BBB and Defendants were so intimately involved in the fraudulent scheme that BBB's Mozayeni openly *celebrated* the fraud in his e-mail to Leschly.

### **BBB and SKI Enter into Additional Secret Agreements**

78. Unbeknownst to EGT, and in furtherance of Defendants' unlawful scheme, BBB entered into additional secret agreements with SKI in November 2011. As discussed in greater detail below, TRV, Leschly, BBB, and SKI kept these agreements hidden for years, until Leschly disclosed their existence in a public court filing in October 2016.

79. On November 21, 2011, with the knowledge and/or participation of TRV and Leschly, SKI and BBB secretly executed an "Option Agreement" whereby SKI granted to BBB the right to obtain an exclusive license to the proprietary information developed by EGT, including information necessary to make, use, and sell the EGT Vector. On the same day, November 21, 2011, SKI and BBB also entered into a "Collaboration Agreement," under which SKI and BBB

agreed to “work together to discover and develop ‘next generation’ lentiviral vectors for the treatment of beta hemoglobinopathies.” Both agreements were signed by Leschly on behalf of BBB. Neither the Option Agreement nor the Collaboration Agreement were disclosed to EGT.

80. The Option and Collaboration Agreements executed by SKI and BBB memorialized what Leschly, TRV, and BBB had been planning for many months. They effectively permit BBB to “shut down” EGT and the EGT Vector by withholding funding for development and commercialization of the EGT Vector. The Option Agreement gives BBB a “fully paid-up and royalty-free” license to use the EGT Vector and know-how for “internal research purposes.” This provision, in combination with a corresponding provision in the Collaboration Agreement, permits BBB to incorporate all of the intellectual property associated with the EGT Vector into the Genetix Vector, in BBB’s discretion.

81. Significantly, BBB’s right to use the EGT Vector under the Option Agreement is exclusive. The Option Agreement provides that SKI “will not initiate, respond to . . . or participate in any way in any discussions or diligence regarding, or accept any proposal for, any license or option or other right in or to” the EGT Vector. In other words, the Option Agreement gives BBB an exclusive, royalty-free license to use the EGT Vector, and prevents SKI from collaborating with any other commercial source to fund clinical trials or to manufacture the EGT Vector.

82. Like the Option Agreement, the Collaboration Agreement grants BBB an “irrevocable, royalty-free” license to use the EGT Vector for “internal research purposes.”

83. The Collaboration Agreement also provides that Sadelain, who had worked closely with EGT on the EGT Vector since the early 2000s, would “work exclusively with BBB” in connection with the development of a vector to treat sickle cell disease and thalassemia. Sadelain was the person most knowledgeable about the EGT Vector; indeed, the EGT Vector is based on

Sadelain's patented technology. Even though SKI had purportedly agreed with EGT in June 2011 to "diligently proceed with clinical trials" and to pay EGT 50% of revenues derived from the EGT Vector, the Collaboration Agreement's exclusivity provision *prohibits* Sadelain from working on the EGT Vector that Sadelain invented.

84. Under the terms of these agreements, TRV, BBB, and Leschly could effectively take EGT's technology and put the EGT Vector "on the shelf." The agreements gave BBB discretion to provide funding to SKI while also prohibiting SKI from obtaining alternative financing.

#### **BBB Hides Its Secret Agreements with SKI in SEC Filings**

85. BBB, Leschly, and TRV were so intent on concealing BBB's secret partnership and conspiracy to defraud EGT that they were willing to withhold from the SEC and the public the material fact that it had entered into multiple agreements with SKI in connection with the EGT Vector.

86. In a June 18, 2013 Prospectus filed with the SEC in advance of BBB's initial public offering, for example, BBB failed to disclose its agreements with SKI. Incredibly, the only mention of SKI appears in a section of the Prospectus describing BBB's "Competition." The Prospectus states as follows:

#### **Competition**

The biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprietary products. . . . There are also several different groups developing gene therapy approaches for  $\beta$ -thalassemia. Some of these groups use a similar *ex vivo* autologous approach, but make use of different vectors and different cell processing techniques. *These include: Memorial Sloan Kettering*, which received approval for its IND in 2012, and is actively recruiting for a Phase I/II gene study. . . . (emphasis added).

The Prospectus never discloses the material fact that BBB had entered into multiple agreements with SKI, including the Option and Collaboration Agreements, for one of the primary treatments under development at BBB. Instead of identifying SKI as a partner or collaborator, BBB's Prospectus describes SKI as a *competitor*.

87. BBB repeated this omission in subsequent SEC filings, including in Annual Reports (Form 10-Ks) and Registration Statements (Form S-1) through the end of 2016.

88. BBB's failure to disclose these material facts in its SEC filings prevented EGT from uncovering BBB, TRV, and Leschly's scheme to shelve the EGT Vector and eliminate EGT as a competitor.

#### **Leschly Instructs Stock Analysts to Conceal Secret Agreement with SKI**

89. In addition to failing to disclose in SEC filings the material fact that BBB had entered into an agreement with SKI, Leschly went so far as to instruct others, including stock analysts, to participate in Defendants' concealment. On November 16, 2012, a stock analyst from The Capital Group contacted Leschly about a press release concerning EGT and asked, "A direct competitor, no?" In response, Leschly stated:

All good. We have an option on the program if we want it . . . and we are working with them to build 'best of breed' as part of a *lock them up strategy*. . . . Please keep this very tight. *We did not disclose on purpose* (emphasis added).

90. Similarly, in an e-mail on March 13, 2013, Leschly advised a venture capital firm, Forbion Capital Partners, that Forbion "SHOULD NOT respond" to EGT's Girondi and that "We have a 'DO NOT RESPOND' policy for anyone he approaches at bluebird." At Leschly's instruction, Forbion agreed to send an e-mail "to the whole partnership" advising them not to respond to EGT. Leschly's instructions to outside investors – coupled with BBB's DO NOT RESPOND policy – furthered Defendants' unlawful concealment and, coupled with Defendants'



other acts to conceal its fraud, made it impossible for EGT to discover Defendants' secret dealings with SKI.

#### **Leschly and TRV Hide BBB's Secret Agreement with SKI From EGT**

91. On countless occasions between 2010 and 2015, Girondi communicated with Leschly about opportunities for EGT to partner with BBB. Despite literally dozens of e-mails, telephone calls, and in-person meetings – and with the knowledge that EGT had worked for a decade with SKI and Sadelain to develop the EGT vector – neither Leschly nor anyone at BBB or TRV ever disclosed the existence of BBB's secret agreements with SKI.

92. Leschly took repeated steps to conceal Defendants' fraud because he knew that EGT would take action. In one internal e-mail, Leschly complains about Girondi contacting him, announces his “plan to continue to ignore” Girondi, and acknowledges that “when [Girondi] finds out about our relationship with [SKI] he is going to go crazy.”

#### **Limited Clinical Trials of the EGT Vector Return Positive Results**

93. After signing the 2011 Agreement, SKI treated only four patients with the EGT Vector. By contrast, upon information and belief, approximately 40 patients were treated with the inferior Genetix Vector.

94. Patients treated with the EGT Vector responded favorably during clinical trials. The EGT Vector significantly reduced a patient's need for blood transfusions and extended life expectancy. Notwithstanding these encouraging results, in a letter dated February 18, 2015, SKI advised EGT that it was no longer actively pursuing exploitation of the EGT Vector. As SKI's Viviane Martin suspected back in May 2010 when first contacted by Leschly, the EGT Vector had been put “on a shelf,” and EGT – BBB's only competitor – had been eliminated.

95. Meanwhile, the Genetix Vector is proceeding to market with the benefit of EGT's know-how and trade secrets, and the rights and property that EGT was fraudulently induced to transfer to SKI.

#### **EGT Learns about BBB's Secret Agreement with SKI**

96. In September 2016, EGT issued a subpoena to Leschly in connection with the lawsuit filed by EGT against SKI in New York. The subpoena commanded deposition testimony from Leschly.

97. Leschly responded by publicly filing a motion to quash the subpoena. The publicly filed motion stated that BBB entered "into a separate contract with SKI." The motion to quash also acknowledged that EGT is BBB's "main competitor."

98. The filing of the motion to quash was the first time that EGT learned that SKI and BBB had entered into an agreement.

99. At all relevant times, Leschly, TRV, and BBB actively participated, directed, and guided SKI's unlawful conduct in its dealings with EGT.

#### **Price-Gouging by BBB**

100. Today, the only "product" listed on BBB's website is "Zynteglo," which is a drug therapy treatment for certain transfusion-dependent thalassemia patients 12 years and older. Zynteglo has been approved for patients in the European Union, Iceland, Liechtenstein, and Norway. It does not heal the sickest thalassemia patients.

101. In June 2019, with a monopoly on gene therapy treatment for sickle cell disease and thalassemia, BBB announced that it would charge an astonishing \$1.8 million per patient for Zynteglo. BBB's ability to price-gouge is the direct result of eliminating EGT as a competitor. BBB's nearly \$6 billion market cap is similarly a product of its misappropriation of EGT's

proprietary intellectual property and the free rein BBB has enjoyed to develop a gene therapy *with* EGT's intellectual property but *without* EGT as a competitor.

### **Harm to EGT Resulting from Fraudulent Scheme**

102. After Leschly, TRV, and BBB succeeded in their unlawful efforts to “shut down” EGT and misappropriate EGT's technology, they had virtually no competition in the field of gene therapy for sickle cell disease and thalassemia. In 2013, BBB had a market cap of under \$1 billion; today, BBB has a market cap of nearly \$6 billion.

103. EGT has been harmed by the acts undertaken by Defendants to destroy EGT's business. Defendants' hidden scheme resulted in the conversion of confidential information developed by EGT over decades. EGT invested tens of millions of dollars in the development of its technology.

104. Defendants also damaged EGT by intentionally interfering with EGT's contractual and advantageous business relationship with SKI. Defendants' unlawful interference caused EGT to lose the benefit of its agreement with SKI – a benefit worth hundreds of millions of dollars.

105. Additionally, EGT is entitled to the financial gain received by Defendants as a result of this intentional interference with EGT's relationship with SKI. In 2010, TRV invested \$35 million in Genetix. Three years later, after successfully misappropriating the EGT Vector and “shutting down” EGT, stock analysts at Cowen and Company reported that they “view beta thalassemia and sickle cell disease as \$500MM+ market opportunities that could support regulatory filings in 2019-2020.” Based on its analysis, Cowen and Company concluded that the value of BBB's thalassemia business unit was \$460 million.

106. Since 2012, Leschly has personally received more than \$55 million in total compensation in his role as CEO of BBB (in 2018 alone, Leschly's total compensation was just

shy of \$24 million). This excessive executive compensation is a direct result of Leschly's and TRV's scheme to wrest control of the EGT Vector from EGT and eliminate EGT as a competitor.

107. Moreover, since 2014, Leschly has sold more than 1 million shares of BBB valued at more than \$80 million.

108. In view of (a) the tens of millions EGT invested in the development of the EGT Vector; (b) TRV's \$35 million investment in the failing Genetix in 2010; (c) the fact that EGT's development of a gene therapy was years ahead of Genetix/BBB at the time Leschly, TRV, and BBB misappropriated EGT's trade secrets and eliminated EGT as a competitor; (d) industry analyst data reporting "\$500MM+ market opportunities" for BBB; (e) BBB's current market cap of approximately \$6 billion, notwithstanding that BBB has a *single* drug on the market in the United States; and (f) BBB's recent announcement to charge patients \$1.8 million for Zynteglo, EGT has suffered damages for which Defendants are jointly and severally liable totalling at least \$1 billion.

## **CAUSES OF ACTION**

### **COUNT I**

#### **(Tortious Interference with Contractual Relations)**

109. EGT repeats and realleges each of the foregoing allegations of the Complaint.

110. EGT had a contractual relationship with SKI.

111. Defendants were aware of EGT's contractual relationship with SKI and, through improper motives and means, knowingly induced SKI to breach its contractual obligations to EGT.

112. As a direct and proximate result of the Defendants' unlawful conduct, EGT has been damaged and continues to be damaged.

**COUNT II**

**(Tortious Interference with Advantageous Business Relations)**

113. EGT repeats and realleges each of the foregoing allegations of the Complaint.

114. EGT had beneficial business relationships with SKI.

115. Defendants were aware of EGT's beneficial business relationships and, through improper motives and means, knowingly interfered with these relationships.

116. As a direct and proximate result of the Defendants' unlawful conduct, EGT has been damaged and continues to be damaged.

**COUNT III**

**(Misappropriation of Trade Secrets)**

117. EGT repeats and realleges each of the foregoing allegations of the Complaint.

118. EGT possessed trade secrets and confidential business information, including the intellectual property, development data, regulatory information, and know-how described above.

119. EGT took reasonable steps to preserve the secrecy of such trade secrets and confidential business information. EGT transferred this information to SKI as part of a confidential relationship under which SKI had a duty not to improperly disclose or use the information.

120. At all relevant times, Defendants had actual knowledge and constructive notice of EGT's ownership of its trade secrets and confidential information and SKI's duty not to disclose or use EGT's trade secrets and confidential information.

121. Defendants obtained EGT's trade secrets from SKI and personally benefitted from EGT's trade secrets despite knowledge that SKI's transfer of such information to Defendants breached SKI's obligations to EGT. In doing so, Defendants used improper means, in breach of a confidential relationship, to acquire, use, and personally benefit from EGT's trade secrets.

122. As a direct and proximate result of the Defendants' unlawful conduct, EGT has been damaged and continues to be damaged.

**COUNT IV**  
**(Violations of Mass. Gen. Laws ch. 93, §42)**

123. EGT repeats and realleges each of the foregoing allegations of the Complaint.

124. EGT possessed trade secrets and confidential business information, including the intellectual property, development data, regulatory information, and know-how described above.

125. EGT took reasonable steps to preserve the secrecy of such trade secrets and confidential business information. EGT transferred this information to SKI as part of a confidential relationship under which SKI had a duty not to improperly disclose or use the information.

126. At all relevant times, Defendants had actual knowledge and constructive notice of EGT's ownership of its trade secrets and confidential information and SKI's duty not to disclose or use EGT's trade secrets and confidential information.

127. Defendants acquired, used, and personally benefitted from EGT's trade secrets and confidential business information through improper means and used EGT's trade secrets without EGT's consent in violation of Mass. Gen. Laws ch. 93, §42.

128. As a direct and proximate result of the Defendants' unlawful conduct, EGT has been damaged and continues to be damaged.

**COUNT V**  
**(Civil Conspiracy)**

129. EGT repeats and realleges each of the foregoing allegations of the Complaint.

130. Defendants knowingly participated in the wrongdoing described above in unison and in a concerted agreement and common design to take these actions against EGT, its interest, and its relationships.

131. Further, acting in unison, Defendants perpetrated this concerted wrongdoing with the intent and effect of utilizing the resulting power of coercion held over EGT that they would not have otherwise had if acting independently.

132. As a direct and proximate result of the unlawful conduct of Defendants, EGT has been damaged and continues to be damaged.

**COUNT VI**  
**(Unjust Enrichment)**

133. EGT repeats and realleges each of the foregoing allegations of the Complaint.

134. Defendants have received significant benefits from EGT.

135. Defendants have failed to provide EGT with any reimbursement of such amounts or other offsetting counter-performance.

136. As a result, Defendants have been unjustly enriched at the expense of EGT such that permitting Defendants to retain the benefit of the property of EGT is against equity and good conscience.

**COUNT VII**  
**(Violations of Chapter 93A)**

137. EGT repeats and realleges each of the foregoing allegations of the Complaint.

138. Defendants are engaged in trade or commerce.

139. Defendants' acts and omissions constitute unfair and deceptive acts or practices in violation of G.L. c. 93A, §11 ("Chapter 93A").

140. Defendants' violations of Chapter 93A were knowing and willful.

141. Defendants' wrongful conduct occurred primarily and substantially within Massachusetts.

142. As a direct and proximate result of Defendants' unfair and deceptive acts and practices, EGT has been damaged and continues to be damaged.

**PRAYER FOR RELIEF**

**WHEREFORE**, EGT respectfully requests that the Court enter judgment as follows:

(a) As to Count I, enter judgment in favor of EGT and award EGT monetary damages in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law;

(b) As to Count II, enter judgment in favor of EGT and award EGT monetary damages in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law;

(c) As to Count III, enter judgment in favor of EGT and award EGT monetary damages in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law;

(d) As to Count IV, enter judgment in favor of EGT and award EGT monetary damages in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law;

(e) As to Count V, enter judgment in favor of EGT and award EGT monetary damages in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law;

(f) As to Count VI, enter judgment in favor of EGT and award EGT monetary damages in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law;

(g) As to Count VII, enter judgment in favor of EGT and award EGT monetary damages, including treble damages, in an amount to be determined against Defendants, together with interest, costs, attorneys' fees, and other amounts under applicable law.

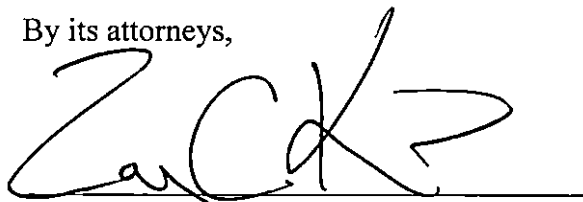


**DEMAND FOR JURY TRIAL**

EGT hereby demands a trial by jury on all counts so triable.

ERRANT GENE THERAPEUTICS, LLC,


By its attorneys,



Mark A. Berthiaume (BBO#041715)  
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September 25, 2019

SUFFOLK SUPERIOR COURT  
CIVIL CLERK'S OFFICE  
2019 SEP 25 1 P 4: 01  
MICHAEL JOSEPH DONOVAN  
CLERK / MAGISTRATE



2019  
1832

# Exhibit F

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**From:** Ewing, Alexandra M. <Ewing@rlf.com>  
**Sent:** Thursday, February 10, 2022 2:38 PM  
**To:** Wilson, Samantha; Cottrell, Fred; 'robert.gunther@wilmerhale.com'; 'christopher.noyes@wilmerhale.com'; 'Isley.Gostin@wilmerhale.com'  
**Cc:** 'Wanda French-Brown'; Lenore Horton; Mary Jean Kim; Alex Cavazos; Crystal Law; Gaza, Anne Shea; jblumenfeld@morrisnichols.com; jtigan@morrisnichols.com; Dittmann, Eric W.; Bennett, Joshua M.; Yusem, Max; Ho, Krystina L.; Modi, Naveen  
**Subject:** RE: Errant Gene Therapeutics, LLC v. Bluebird Bio, Inc., et al., C.A. No. 21-1478-RGA (D. Del.)

**Follow Up Flag:** Follow up  
**Flag Status:** Flagged

Samantha,

At this point—before being named in a complaint and before seeing the claims in EGT’s proposed amended pleading—Sloan Kettering takes no position on whether it is a necessary party in this case. But Sloan Kettering maintains that this suit, like the suit that EGT filed against Sloan Kettering in the Southern District of New York, is improper, at least because the claims were released by the 2020 agreement and because the parties agreed to submit any dispute relating to the 2020 agreement to arbitration. Sloan Kettering will not join EGT’s complaint as a co-plaintiff because Sloan Kettering does not believe there is a good-faith basis for EGT’s claims. If EGT names Sloan Kettering as a defendant under Rule 19(a)(2), *see* 7A Charles Alan Wright & Arthur R. Miller, Federal Practice and Procedure § 1605 (3d ed. Apr. 2021 update), Sloan Kettering reserves the right to challenge whether it is a necessary party (and raise any other defenses) after it is served with a pleading.

Best,  
Ally

Alexandra M. Ewing  
Richards, Layton & Finger, P.A.  
One Rodney Square  
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302-651-7898 (direct dial)  
[Ewing@rlf.com](mailto:Ewing@rlf.com)

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**From:** Wilson, Samantha <SWilson@ycst.com>  
**Sent:** Monday, February 7, 2022 12:17 PM

**To:** Cottrell, Fred <Cottrell@RLF.com>; Ewing, Alexandra M. <Ewing@rlf.com>; 'robert.gunther@wilmerhale.com' <robert.gunther@wilmerhale.com>; 'christopher.noyes@wilmerhale.com' <christopher.noyes@wilmerhale.com>; 'Isley.Gostin@wilmerhale.com' <Isley.Gostin@wilmerhale.com>  
**Cc:** 'Wanda French-Brown' <wfrenchbrown@loeb.com>; Lenore Horton <lenore@hortonlegalstrategies.com>; Mary Jean Kim <mjkim@loeb.com>; Alex Cavazos <acavazos@loeb.com>; Crystal Law <crystallaw@loeb.com>; Gaza, Anne Shea <agaza@ycst.com>; Wilson, Samantha <SWilson@ycst.com>  
**Subject:** Errant Gene Therapeutics, LLC v. Bluebird Bio, Inc., et al., C.A. No. 21-1478-RGA (D. Del.)

**\* EXTERNAL EMAIL \***

Counsel,

I write on behalf of Errant Gene Therapeutics ("EGT") regarding the above-referenced Delaware lawsuit to seek your clients' position as to EGT's standing in that case.

As you know, EGT has sued the defendants for infringement of U.S. Patent Nos. 7,541,179 and 8,058,061, to which your clients, Memorial Sloan Kettering Cancer Center ("MSKCC") and the Sloan Kettering Institute for Cancer Research (together, "Sloan Kettering"), granted EGT an exclusive commercial license. On January 14, 2022 defendants filed a motion to dismiss EGT's First Amended Complaint, including for lack of standing.

While EGT is confident that the First Amended Complaint can withstand the motion to dismiss, EGT has endeavored to resolve the motion through the filing of a proposed second amended and supplemental complaint ("SAC") setting forth, *inter alia*, additional facts regarding EGT's standing to sue in its own right. However, defendants have indicated that they still intend to make similar standing objections to the proposed SAC. EGT therefore asked whether defendants will consent to joining the assignee, MSKCC, to the lawsuit, thereby eliminating any possible basis for a renewed motion to dismiss based on lack of standing and/or failure to join a necessary party. Those discussions are ongoing but defendants have asked if MSKCC will voluntarily join the Delaware lawsuit.

Because EGT has standing to bring the Delaware lawsuit in its own right under well-settled case law, EGT has only raised the possibility of a Fed. R. Civ. P. 19 motion to join because of defendants' intent to renew their motion to dismiss. But EGT also does not want to burden Judge Andrews with unnecessary motion practice. EGT therefore requests Sloan Kettering's position as follows:

1. Please confirm Sloan Kettering agrees that MSKCC is not a necessary party under Fed. R. Civ. P. 19 because EGT has standing to sue in its own right.
2. If Sloan Kettering is unable or unwilling to so confirm, please confirm MSKCC will consent to join the Delaware lawsuit as a co-plaintiff, without objection to jurisdiction and/or venue.

Please respond no later than Thursday, February 10. We are available to discuss as needed.

Regards,  
Samantha



**Samantha G. Wilson, Associate**  
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